SCIENTIFIC OPPORTUNITIES AND PUBLIC NEEDS: BALANCING NIH'S PRIORITY SETTING PROCESS

HEARING

BEFORE THE

SUBCOMMITTEE ON HEALTH

OF THE

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HOUSE OF REPRESENTATIVES

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SCIENTIFIC OPPORTUNITIES AND PUBLIC NEEDS: BALANCING NIH'S PRIORITY SETTING PROCESS

WEDNESDAY, JUNE 2, 2004

HOUSE OF REPRESENTATIVES,
COMMITTEE ON ENERGY AND COMMERCE,
SUBCOMMITTEE ON HEALTH,
Washington, DC.

The subcommittee met, pursuant to notice, at 2:10 p.m., in room 2123, Rayburn House Office Building, Hon. Michael Bilirakis (chairman) presiding.

Members present: Representatives Bilirakis, Greenwood, Shimkus, Pitts, Rogers, Barton (ex officio), Brown, Waxman, Stupak, Green, Strickland, and Capps.

Staff present: Chuck Clapton, majority counsel; Cheryl Jaeger, majority professional staff; Jeremy Allen, health policy coordinator; Eugenia Edwards, legislative clerk; and John Ford, minority counsel.

Mr. BILIRAKIS. The hearing will come to order. Good afternoon. Today's hearing, which we have entitled "Scientific Opportunities and Public Needs: Balancing NIH's PrioritySetting Process," is the fifth hearing that this subcommittee has held during this Congress in order to highlight research activities at the NIH. These bipartisan hearings have educated members and others about the work that the NIH is doing so we can access how to help the support and research entity better meet its stated mission.

In many respects all of the hearings that we have previously conducted have been leading up to today's hearing. Now that members understand hopefully what the NIH is doing, we need to understand how they choose what research to conduct and how that research is funded.

The NIH is the world's leader in conducting important research that will unlock critical information and lead to discoveries beneficial to patients suffering from many diseases.

Over the years the NIH has seen significant increases in its funding and the development of many new programs.

What began as a one-room laboratory of hygiene in 1887 now consists of 27 Institutes and Centers that have been created in response to legislative or executive decisions.

When an institute is created, it has typically been provided a separate annual budget from Congress. With this individual budget comes the responsibility of setting priorities and making budget decisions within the Institute's domain.

I am very interested, as we all are, I think, in hearing from today's witnesses about how they balance setting priorities in their institute and then how they coordinate those decisions across the entire NIH.

The priority setting process at NIH and within every individual institute has drawn questions, God knows, from Members of Congress as well as patient advocacy groups and others. I believe that much of this criticism has arisen because the priority setting process is extremely complicated, especially the grant approval process, and because NIH lacks transparency in many of their decision-making procedures.

Hopefully today's hearing will give members an opportunity to really understand what criteria is used to determine which grants are funded and why.

To address these issues, we have an excellent panel of witnesses before us. I would like to welcome them.

First, Dr. Elias Zerhouni, Director of the NIH, who is here today. Doctor, you have been so gracious with your time over the past year. You have been here before us, what, the second or third time, I think, over the past few months, and we certainly appreciate all of your efforts to assist the subcommittee's work on NIH.

In addition to Dr. Zerhouni, we have three very distinguished institute directors: Dr. Anthony Fauci, who has been the Director of the National Institute of Allergy and Infectious Diseases since 1984. Dr. Fauci, we look forward to hearing about your experience over these years.

Dr. Andrew von Eschenbach, the Director of the National Cancer Institute; and Doctor Nora Volkow, the Director of the National Institute of Drug Abuse, will also be able to discuss the unique challenges they face. Thank you both for coming, and I know that all of you have had to rearrange your schedules to be here this afternoon, and you are some of the busiest people in our world, and we certainly appreciate you taking the time.

In addition to hearing your individual testimony, I would also like to initiate a dialog. I would like to see a dialog between and among each of you as to how you have been working together to ensure that the grants elected for funding measure up in importance to not only your institute but the entire NIH and the degree that you have been effective in your collaborations.

Again, thanks for being here. I know that we have all been very, oh, sometimes quizzical, if you will, concerned, whatnot, in terms of how the NIH allocates their resources for research. And I would hope that we could keep our opening statements as brief as possible so we can give these gentlemen and lady an opportunity to explain all of that to us.

I would now yield to the ranking member of the subcommittee, the gentleman from Ohio, for an opening statement.

Mr. BROWN. Thank you, Mr. Chairman. I especially appreciate Dr. Zerhouni being here and his terrific work, the terrific work of all three of you on the panel. And I want to single out Dr. Fauci for his excellent work on infectious disease and what that means for Americans, and especially in the developing world, especially on tuberculosis and malaria and all that you have done that way.

I think all of us receive letters every day from constituents urging support for specific—to deal with specific illnesses, for cancer research, Alzheimer's, diabetes, arthritis, lupus, cystic fibrosis. Every one of these health conditions and thousands of others compromise equality and length of our constituents' lives. It is incumbent upon Members of Congress to ensure that NIH resources are allocated in a manner that is reasoned, that is efficient and fair, but this responsibility doesn't exist in a vacuum. We have a requisite obligation to ensure ample funding overall for NIH.

A memo recently leaked to the press indicates that the President plans to cut $600 million from NIH in 2006 to make room for his continued request for tax cuts. Let me place that in context. We have been increasing NIH funding. Mr. Bilirakis has played a major role in the doubling of NIH funding, as we all have bipartisanly, but we have been increasing funding by a billion or so each year.

I am sure that, Dr. Zerhouni, that these cuts didn't come from your desk, and I don't believe the Chairman or my fellow colleagues on either side of the aisle would support that kind of request from the President to make these kinds of cuts in the National Institutes of Health, but we need to be aware that NIH cannot evolve without the resources to do so. Prioritizing research doesn't mean anything if you can't fund it, obviously.

You all don't have an easy job, nor does Congress. Both of us walk a fine line in terms of how much influence to exert on the general direction of medical progress and the priority given to researching various diseases.

Should we invest in diseases that are most prevalent, the most deadly or disabling to Americans, to the poor in the developing world, or for those which have the greatest chance of finding a cure? Should we focus more dollars on the here-and-now concrete answers to concrete questions or in paradigm shifts such as those represented by human genomics?

As a rule, I think most of us, and I will speak obviously for myself, have tried to steer clear of any effort by Congress to compromise the flexibility NIH has to allocate the tens of billions of dollars it received. However, a few years ago I made an exception. I raise the example here because it illustrates, I think, three points:

One, that it is in fact important for NIH to revisit and refind a way that allocates funding on a regular basis.

Second, that Congress as a representative of the public must continue to play an oversight role and challenge NIH to respond to public concerns about the agency's funding decisions.

And third, what if NIH is not sufficiently responsive to the public, it doesn't matter how the agency sets its priorities, because those priorities will almost there, by definition, be wrong.

In 2001 I cosponsored legislation that required NIH to pay more attention to Duchenne muscular dystrophy. Specifically, NIH was to expand and intensify research related to Duchenne and other forms of MD and support centers of excellence that would foster external muscular dystrophy research. It required HHS to establish an interagency committee to coordinate all Federal muscular dystrophy programs and activities.

I joined this effort because—and this is one of the few times it has happened in a major way I think—it was abundantly clear that Duchenne has somehow fallen through the cracks at NIH, despite the fact it is the world's most prevalent childhood killer. Still today, resources devoted to this disease represent less than .0005 percent of the NIH budget.

The MD Care Act was signed into law by President Bush in 2001. The CBO scored it at $56 million in new spending over 5 years. My understanding is NIH has barely begun to fund the research in surveillance programs established under the new law, and it has been 3 years, and that the CDC surveillance program is off to a sluggish start.

Congress is very reluctant, as I said earlier, and as Mr. Bilirakis has said in the past, to be prescriptive in appropriations report language with NIH, but the fiscal 2004 Labor-HHS appropriations bill calls for full funding in this fiscal year of three additional centers of excellence.

I understand NIH has solicited proposals for, "2 or 3 centers," and the funding won't be released until well in the next year. The coordinating committee created under the new law has met only twice since the bill's enactment.

It is my understanding the NIH has contributed to the funding of only one clinical trial focused on Duchenne muscular dystrophy. NIH has publicly stated it has received only three clinical trial requests for Duchenne over the years. However, scientists interested in this condition have told us that that number is not accurate.

The NIH in the past, certainly, and in other diseases is not just a passive recipient of research proposals. The agency also obviously solicits proposals from the research community. It is my understanding that no such solicitation has been made in regard to Duchenne, even though established clinical trial and research center networks exist and important research opportunities have been identified.

I am confident, Dr. Zerhouni, in your dedication and sincere interest in moving forward in NIH and the public interest as you always have, but I also contend that NIH can't ignore concerns raised by the public in Duchenne and other things.

Mr. Chairman, if the public loses confidence in NIH, it doesn't matter how we set our priorities and how you set your priorities, Congress will be unable to secure the funding ultimately needed to sustain this crucial agency, especially if the President gets his way to slash spending in this agency.

We mustn't let that happen.

Mr. BILIRAKIS. I thank the gentleman.

The chairman of the full committee, Mr. Barton, good to have you.

Chairman BARTON. Thank you, Mr. Chairman. And I have a formal statement that I would ask unanimous consent that it be in the record.

Mr. BILIRAKIS. Without objection, it will be.

Chairman BARTON. I am going to speak extemporaneously, because I want to try to be very clear. This is the fifth hearing that this subcommittee has held on the structure or the goals of NIH,

and Dr. Zerhouni has been cooperative in all of these hearings, and I want to thank you.

Today we want to look at the mission statement of how NIH sets its priorities. That is the official title, and I want to thank our three institute directors for coming.

I had lunch with Dr. Von Eschenbach not too many weeks ago, with former Congressman Archer, and it is good to see you again.

But our oversight subcommittee is also holding hearings on NIH, and the oversight function of this committee, and the Congress is to kind of serve as a watchdog, and so we are kind of on a dual track here. We are in the oversight function looking at the way certain things are being done, and Dr. Zerhouni is cooperating in that, but this subcommittee is looking at the general structure of NIH and how we can maybe reorganize, reprioritize, reform to make it better.

There are certain things that the committee is not at all concerned about. We are not concerned about your peer review process. You all have thousands of reviewers that do tens of thousands, probably, of peer reviews every year, and I think that is a good system. We are concerned that the NIH as it has evolved—we now have 27 institutes and centers and they have kind of grown up serendipitously.

We have one director appointed by the President, Dr. Zerhouni right now, and he doesn't have a lot of control over the centers and the institutes. And I would like to see if we can, on a bipartisan basis, through these hearings come up with a legislative package to reauthorize NIH. Most of the programs at NIH have not been reauthorized in a number of years, and that is a lack of discipline on the committee. That is not a problem in NIH. That is our problem. We have not reauthorized your functions, and we have thrown that on the appropriators; in this case, Chairman Ralph Regula's appropriation subcommittee.

So what we want to do in this subcommittee, with Mr. Bilirakis's leadership and Mr. Brown's leadership, is see if we can't work on a bipartisan basis to come up with some legislative reforms that make it easier for NIH to do its function. We are not opposed to the function. We are not opposed to using the best scientific brains in the world to try to find cures and treatments for all the many diseases and afflictions that you folks deal with. But we are also not just going to turn a blind eye and say, you know, business as usual is okay, because the dollars are too big and the consequences are too big. And, quite frankly, the assets at the disposal of NIH are significant, and if we can channel them in a more comprehensive, coordinated fashion, we are going to do great deeds in the years ahead. So that is what these hearings are about.

So I know it is—you know, we are beginning to see in the press, because of what is happening on the oversight subcommittee, you know, that the Congress is out to get NIH or—nothing could be further from the truth. You know, it is just the opposite. We want the most effective state-of-the-art NIH for the 21st century, that gets the biggest bang for the taxpayer bucks and the private sector dollars that are coordinated with what NIH does. That is what these hearings are about, and I want to thank Chairman Bilirakis for

holding them. And our goal is to have an NIH reauthorization package ready to move through this committee in this Congress.

[The prepared statement of Hon. Joe Barton follows:]

PREPARED STATEMENT OF HON. JOE BARTON, CHAIRMAN, COMMITTEE ON ENERGY AND COMMERCE

Thank you, Chairman Bilirakis, for holding this hearing today. I am pleased that the Energy and Commerce Committee continues to invest so much time and energy in reviewing the operation of our most important public health agencies.

Today's hearing is the fifth in a series that examines different aspects of the National Institutes of Health. The more I learn in these hearings, the more concerned I become about the existing NIH priority-setting process. In particular, I am troubled by the relative lack of authority possessed by the Director to set priorities and manage the research portfolio of the entire agency.

Over the years, the organizational structure of NIH has been arbitrarily expanded. This organizational structure largely determines the priority-setting process at NIH. What we are here to figure out is whether or not the current priority-setting structure at NIH is adequate to meet the nation's medical research needs. Congress needs to be able to evaluate if funding allocation decisions are made on the basis of the best assessment of scientific opportunities and equity, in light of existing public health priorities.

Let me be clear: we are not holding this hearing today to argue that some of the science conducted by NIH lacks merit. The scientific peer review process at NIH works remarkably well considering the volume of grant applications reviewed each year. I'm certainly not interested in picking and choosing among the thousands of diseases that afflict Americans, to determine who should be the "winners" and "losers" of NIH funding. But to ignore the directions that Congress has already made in dictating how research priorities are set at NIH would be a mistake.

Dr. Zerhouni, given the current problematic NIH structure, you should be commended for your leadership. Your efforts to design an NIH Roadmap to link all of the research activities at the 27 separate institutes and centers towards shared research goals is a particularly important step.

In addition to Dr. Zerhouni, the Committee is privileged today to hear from three Institute directors, who are also some of the top scientists in the world. Several of them rescheduled travel and other commitments to attend today's hearing, which underscores the importance of the topic. Dr. Fauci, for over two decades your leadership has helped to control the rapid spread of infectious disease and prepare our country to respond to potential bioterrorist attacks. In addition, Dr. Von Eschenbach, and Dr. Volkow are nationally recognized experts in the fields of cancer care and drug abuse, respectively. I look forward to today's testimony and again want to thank all of the witnesses for rearranging their schedules to attend the hearing.

Mr. BILIRAKIS. The Chair thanks Chairman Barton.

Without objection, by the way, the opening statement of all members of the committee will be made a part of the record, and now I recognize Mr. Waxman for an opening statement.

Mr. WAXMAN. Thank you very much, Mr. Chairman.

The NIH is the most important and successful medical research organization in the world. It has produced more cures, more breakthrough treatments, and more hope for millions of patients around the world than any other group of scientists.

I think we can all agree that Congress and NIH must work together to set NIH's research agenda and priorities in the broad sense. Congress, with NIH's guidance, must decide how much to appropriate to the general research areas covered by each of the institutes and centers, and of course Congress must exercise oversight.

After that, however, Congress should step back and allow NIH scientists to decide what specific research projects will produce the greatest gains for humanity. I am increasingly concerned about congressional interference in NIH decisions to fund specific re-

search grants. In making those decisions, NIH employs a rigorous and highly respected peer review process. NIH uses over 11,000 scientific experts, all of which have had—all of whom have had many years of scientific training and are recognized in their fields, to staff 170 peer review panels.

As the members of this subcommittee look into NIH's work, I hope that we will all exercise self-restraint. In the past, some Members of Congress have given in to the temptation to substitute their scientific judgment for that of the peer review process, and I think that is a very perilous activity.

The fact that social conservatives disapprove of certain kinds of sexual behavior or drug use cannot be the basis for deciding whether scientific research on that behavior is worth funding. Funding decisions must instead be based on whether such research will or will not help us learn how to stop the spread of serious diseases and reduce human suffering.

And I am very pleased that Dr. Zerhouni has affirmed both the scientific importance of research on sexual behavior in his continuing support for the peer review process at NIH, and I hope that from this subcommittee that we will have a continuation of the policy to support a process whereby our best scientists pursue the research that they have determined offers the best chance to save many lives.

I know that we are looking at other issues with NIH in the Oversight Subcommittee, and I am part of that subcommittee, and those issues that have been identified ought to be dealt with and we ought to make sure that the NIH lives up to the requirements in the public interest of transparency and accountability. But let's understand NIH is too important and too successful and too valuable for us to in any way interfere with the important scientific work that it is pursuing.

Thank you, Mr. Chairman.

Mr. BILIRAKIS. I thank the gentleman.

Mr. Rogers for an opening statement.

Mr. ROGERS. Mr. Chairman, I am going to yield for questions later.

Mr. BILIRAKIS. Thank you very much.

Mr. Green.

Mr. GREEN. Thank you, Mr. Chairman.

And, again, I would—like the chairman of the full committee, I would like to welcome Dr. Zerhouni back to us, and appreciate your patience.

But I also would be remiss if I didn't welcome my good friend, Dr. Von Eschenbach, who we miss at M.D. Anderson at the Texas Medical Center and your research successes you had in all those years, but I told you earlier I will can our 95 degree temperature and our 95 percent humidity and bring it to you here in DC. Just in case you have missed it so much.

But, Mr. Chairman, I want to thank you and our ranking member for this series of hearings so we can gain knowledge on the important research NIH does, and I thank you for your leadership. The work being performed at NIH has proved invaluable. The groundbreaking research provided a lifeline of hope to countless Americans living with diabetes, cancer and AIDS and many other

illnesses. As a testament to our support for NIH, Congress has completed a 5-year effort to double NIH's funding. Yet as NIH's authorizing committee, it is important for us to understand how the NIH utilizes this funding, how it determines its priorities, and which strategies it sets to meet the goals.

I am particularly interested in NIH's research on diabetes. I represent a district in Texas, it is over 65 percent Hispanic, and nationwide 24 percent of Mexican Americans age 45 to 74 live with diabetes. Mexican Americans are also twice as likely to have the disease as the Anglo population, which is why diabetes treatment and prevention is so important to not only myself but to the district I represent.

Diabetes research and treatment cannot be performed in a vacuum, though, and that is what we know about research in general. Obesity plays such a large role and genetics and race play a role. The disease can lead to a host of other conditions such as kidney failure and cardiovascular disease.

I want to make sure NIH's priority setting process includes a truly collaborative effort—and I know we had this at other hearings—with the myriad of institutes that we have involved, to ensure that we are leaving no stone unturned in the research to find treatments and a cure for this deadly disease.

Stem cell research also provides us with a great promise in finding cures for devastating diseases, diabetes, Alzheimer's and cancer. While it is no secret that NIH's stem cell research is constrained by the President's policy on the issue, I appreciate, Dr. Zerhouni, your candor when you recently wrote that from a scientific perspective, more cell lines may well speed some areas of human embryonic stem cell research. Stem cell research offers a tremendous hope to patients with diabetes, Alzheimer's, Parkinson's and cancer treatment.

Speedy research is exactly what these patients need and this policy is simply a roadblock along the path of research and discovery at the NIH that could lead to cures for many illnesses.

Again, I want to thank the witnesses for being here today and their effort for a number of years, and, again, Mr. Chairman, thank you and our ranking member for your leadership to make sure we continue to have this oversight, and I am glad to hear the Chairman of the committee has the goal of doing the reauthorization, so I am looking forward to that.

Mr. BILIRAKIS. I thank the gentleman.

Mr. Strickland for an opening statement.

Mr. STRICKLAND. Mr. Chairman, I look forward to hearing the witnesses, and I will forego an opening statement. Thank you.

Mr. BILIRAKIS. The Chair thanks the gentleman.

Mrs. Capps.

Mrs. CAPPS. Thank you, Mr. Chairman.

I also want to thank Director Zerhouni, Directors Fauci, Volkow, and von Eschenbach for making yourselves available to us today. We appreciate your time and the fact that you are willing to share your expertise with us.

It is a matter of great pride, I believe, in a bipartisan way that we have over the past few years doubled the funding for NIH, and we in Congress often focus on the benefits that the public gets from

the billions of dollars that are devoted to the NIH. This is a good work that we fund but that you carry out. There is no doubt that this investment has paid off in spades.

But there is an issue that I want to bring up today, hoping that this committee and the NIH could begin to discuss, and that is public access to the fruits of federally funded research. It is complex, and I don't have a black or white answer, but many results of federally funded research are published only in very expensive journals that most of the public has no access to. Many universities, libraries and organizations such as the Howard Hughes Medical Institute, the Welcome Trust, and the Susan G. Coleman Breast Cancer Foundation would like to see this research opened up more fully to the public.

To be sure, there are many questions that have to be answered before this approach is endorsed by Congress or the NIH. I believe that this is an appropriate place to carry out some of this discussion. I hope we can do so in the near future.

And again I want to address an issue that my colleague Mr. Waxman has brought up. I have addressed it before, and maybe it will come up today in some testimony. Some of our colleagues who have little or no scientific or medical expertise—and actually that has nothing to do with that—have raised questions about NIH grants on human sexuality. Congressional oversight is important, but it is critical, I believe, that we be very serious about keeping politics from interfering with science.

NIH was set up to dramatically improve the lives of Americans by increasing the quality and amount of biomedical research conducted, and NIH does this job admirably. We here should not try to micromanage scientists about how to conduct their research, and we should not engage in witch hunts to discourage research into particular areas. I believe there is no question that some Americans engage in self-destructive behavior. If we want to help them to make their lives better, we cannot pretend that that behavior does not exist. So we must come to understand it and its effects on public health in order that it can be addressed more scientifically and more effectively.

I believe that is what scientific research is for. The Congress should not hamper the excellent work of the NIH by trying to impose any kind of ideology onto science.

So I look forward to hearing the testimony that you are going to present to us, yield back the balance of my time.

Mr. BILIRAKIS. The Chair thanks the gentlelady.

I believe that completes the opening statements, and the Chair is grateful for that so we can move right into the witnesses' testimony.

I am going to set 10 minutes for each one of you, if I may, and hopefully you can stay within it. Your written statement is, of course, part of the record. And hopefully you will have an opportunity for dialog among yourselves.

Dr. Zerhouni, please proceed, sir.

STATEMENTS OF ELIAS A. ZERHOUNI, DIRECTOR, NATIONAL INSTITUTES OF HEALTH; ACCOMPANIED BY ANTHONY S. FAUCI, DIRECTOR, NATIONAL INSTITUTE OF ALLERGY AND INFECTIOUS DISEASES; ANDREW C. von ESCHENBACH, DIRECTOR, NATIONAL CANCER INSTITUTE; AND NORA D. VOLKOW, DIRECTOR, NATIONAL INSTITUTE OF DRUG ABUSE

Mr. ZERHOUNI. Thank you, Mr. Chairman. I am pleased to appear in front of your subcommittee and I am pleased to be accompanied today with my esteemed colleagues.

It is an important topic that I think you are addressing, and that is reviewing the NIH research portfolio and discussing our priority setting processes. I have submitted written testimony. What I would like to do is summarize on slides what I think are the salient components of what NIH does to allocate resources to the best extent possible to address the disease burden in our population.

There is no doubt that NIH has been and continues to be at the leading edge of discovery. For example, in the area of infectious diseases we identified the SARS virus in less than 3 weeks. In 1985 it took 3 years to identify the HIV virus, and influenza took 30 years to be identified. This is due to the investment that Congress and the administration have made over the years in NIH.

Clearly, we can also see progress being made in other areas of research besides infectious diseases. This year, for example, we have now discovered over 12 genes which were never suspected before to be the cause of mental illness, including schizophrenia. We are now at the edge or the threshold of understanding how schizophrenia develops in the human population, reminding you that schizophrenia in young adults 25 to 44 years of age is one of the main causes of disability.

We continue to uncover basic principles of human biology. The human genome is just one step in the process of understanding how all of our molecules and cells are organized, and we are continuing to make progress in this area at a very rapid pace, which is being applied across all disease areas.

In addition, I think that life expectancy continues to increase, and it is the result of many factors. Clearly, some of the impact of the research of NIH has changed the landscape of the diseases that we have to deal with and therefore changes the mechanics and the strategies for priority setting in the country.

For example, if you look just at the impact of our research on cardiovascular disease over the past 30 years, it has been remarkable. Mortality from heart disease and stroke has decreased by over 50 percent.

How does that translate in numbers? If you looked at coronary heart disease between 1950 and 2000, and if we had projected in 1970 what the number of deaths would be in 2000, it would be 1,329,000 deaths experienced in 2000 if we hadn't discovered that hypertension was a risk factor, or high cholesterol was a risk factor, or discovered all of the new treatments and therapies that we are using in cardiovascular disease.

So in fact you can say that 815,000 deaths have been prevented in 2000 because of this 30-year investment in cardiovascular disease with this rapid decrease in mortality.

What is the impact of that on life expectancy? Obviously life expectancy increases, but at the same time this acute disease, which used to kill many of our patients very quickly, is transferred into a more chronic acute condition. And if there is one word I can give you as to the scientific priorities of NIH is this transformation of disease from more short-term lethal acute diseases to more long-term chronic conditions, including, for example, cancer where much progress has been made to the extent that survivorship in cancer is unprecedented at this time in history, and we have over 9 million individuals who survived cancer in this country.

So when you look at that, you obviously see a changing environment, and the resource allocations and the budget allocations have to reflect that changing environment in some ways.

Just to go quickly over the NIH budget, there are four ways to look at our budget. First and foremost, you can look at it by mechanism: Do we fund grants, do we fund centers, do we fund contracts?

The second is by research area: How much do we fund in cancer versus infectious disease or pediatric research? The other is to look at how we fund the structures of NIH that have been authorized over the years by Congress, the institutes and centers and offices, and how much funding is in each one of these institutes.

And obviously we can also look at it in terms of scientific effectiveness. In other words, what is the likelihood that we can fund the research enterprise in the country and the capacity that is committed to finding the treatments and prevention strategies that we need?

If you look at that this way, I can show you very quickly that about 85 percent of the NIH budget is spent outside of NIH. About 10 percent of the budget is spent intramurally here in Bethesda. We spend about 4 percent of our dollars on research management and support, which is the administrative expenditures, and about a billion dollars on activities like the National Library of Medicine, education, and so on. But the rest of it is spent on academic institutions, and we support over 212,000 scientists in the country at over 2,800 institutions throughout the country.

So if you wanted to look at the budget by mechanism, where is it intramurally, where is it extramurally, by institutes, you can see the very large range of funded institutes and the levels of funding.

NCI, my dear colleague Dr. von Eschenbach leads, is funded at $4.87 billion. NIAID, under the direction of Dr. Fauci, is $4.4 billion. And you can see the FIC at the bottom right is the Fogarty International Center, which is our international operation, $67 million. So you can see a wide range of allocation of resources for different institutes with different missions and different scope of missions. And this is the complexity that you have to deal with as an institution, and you have to balance that relative to the opportunities in science and the good ideas that come from our scientists.

America is unique in the world, in that it uses a peer review system that is fundamentally based on the ideas of scientists, where investigator-initiated grants come in and we review them and fund a small percentage—about a third of them are funded. So if you look at our likelihood of success to be funded, if you are a scientist in the United States and you came up with a good idea and you came to NIH, in 1996 your funding chances would be 28 percent.

And then it went up during the doubling period, all the way to 32 percent. It remained very competitive. And currently in 2002, 2003, it went to 30 percent, and we project 27 percent for the 2004-2005 period.

Why is it that despite the doubling, the success rate has remained very competitive? Most of the answer is in this graph. We have had more and more scientists drawn to biomedical research because of the long-term commitment and investment of the American people to medical research. In 1996 we had about 23,800 research project applications that year. In 2003 we had 34,700, a huge increase in the research capacity of the United States, which is obviously a good indicator that, A, there is attraction to the field and, B, we remain very competitive in who we grant and how we grant our research dollars.

When you look at priority setting, what I would like to do is show you the entire wheel of how priority setting should occur and occurs in practice. And as the Chairman said, it is a complex process. It is not something that you can explain with one parameter and say this is how we allocate our resources. But at the top of the list is obviously our intent to reduce both disease burden as we know it today, but also potential disease burden as may occur in the future.

A good example is Dr. Fauci's investment in biodefense and the increasing funding in biodefense. I mean, we are doing this not because there is an existing disease burden. We are doing it because of a potential disease burden, and other areas can be illustrated.

So first and foremost, we try to evaluate the disease burden that we are dealing with through a variety of mechanisms. CDC has the National Center for Health Statistics. The National Cancer Institute has a program called Surveillance, Epidemiology and End Results to track cancer progress. Other institutes have different mechanisms. We don't have an established national way of measuring burden for all diseases; however, we do it for the most important ones.

But let's say now that a disease burden is emerging, like obesity, for example, as a new threat, or chronic disease, which we did not experience in the past which are now becoming very important. How do we then adjust the portfolio, adjust the strategy?

First and foremost, we need to have, obviously, a public investment in the area. What is the public investment used for? First, we have to build scientific capacity. You cannot advance in the field of research for any one disease if you do not have the people, the resources, the ideas, the buildings, the laboratories. And that is the first thing that NIH does. A good example of that is what Dr. Fauci will talk to you about. Today he is engaged in building scientific capacity for biodefense research. They are building laboratories across the country at NIH and in different areas.

Once that is done, the next step is to obviously build the research capacity itself in the area of interest. Research in tuberculosis and malaria, versus cancer, versus other areas, requires different disciplines and different combinations of disciplines. That research capacity then hopefully leads to discoveries and breakthroughs that can be then used as new opportunities.

So this is where we see scientific opportunities for therapies occur, and these scientific opportunities then have to be translated, and sometimes this is where I think a lot of the discussion occurs. How much emphasis do we place on scientific opportunity versus why don't we just go and create a lot of clinical trials and do the translation? And the message I like to give to everyone is you can't translate a language you do not understand very well. To the same extent in science, the timing of translation depends on how well you understand the disease process and how much progress you have made at that time and balance the research capacity with the opportunity and with the ability to translate successfully. Hopefully once we have translated that to practice, it is what I call the cycle, lab to life, that essentially underlies every research enterprise that you could analyze at NIH, and you will hear how my colleagues do so within their own mission area. And this is what we need to do also at the NIH level.

So let's talk about how well are we doing relative to the disease burden. Let me show you an independent study that was led by Dr. Cary Gross in 1999 where they evaluated the disease burden, calculated with modern methods of computation of burden, and the modern method is called disability-adjusted life years.

Just to give you a simple example of how that works, if you had a child 1 year old who had a disease and didn't survive, that child would have lost their life expectancy. So that would be 80 years of life lost. If somebody is 98 years old and has a cancer, that is computed really maybe as 2 years of life lost, depending on the actuarial tables. That is the computation that epidemiologists make.

When you look at the diseases studied, the red line is about where the average disease burden versus investment would be. On the left-hand side is the funding in NIH dollars, and the horizontal is the disability-adjusted life-year component.

And as you can see, by and large, the investment is around the mid-life for these diseases. But you will also see outliers, and question the outliers. For example, you can see AIDS at the top of the curve, and this is a common question: Why is AIDS, relative to the current disease burden, higher in funding than other areas? Well, this is a judgment that is made not on the basis of existing disease burden, but potential future disease burden both here and in the world and the potential impact this may have on the economy of the United States.

And I will let Dr. Fauci comment on that. So there are logical ways that we use to correlate, if you will, the disease burden, the scientific opportunity with funding, but those are not precise.

As you can see, there are variations across all diseases.

Now, how do we answer your question, which is, how do we know that NIH has methods that allow it to not only do good peer review but effectively manage its research portfolio? And there are three factors there that we take into account: science, public health requirements, and societal needs. And we get inputs, as you mentioned, from many, many sources, including the public.

One of the things I would like to attach to my testimony is the last report of the Council of Representatives of the National Institutes of Health who studied the transparency issue and how can

we improve our processes to have public input at NIH to a greater degree than we have had in the past.

Mr. BILIRAKIS. Without objection, that will be the case.

[The report appears at the end of the hearing.]

Mr. ZERHOUNI. Thank you, Mr. Chairman.

A good example of adaptation is the example of obesity research. So what mechanisms do we have to make sure that nothing falls through the cracks? Clearly, the burden is going up. CDC is reporting it now as the second preventable mortality after smoking. So we have created a trans-NIH task force last year to review all of NIH's strategies and portfolio. Then the new plan was developed this year. And just to give you an example, we decided to increase funding for obesity research by 10 percent, even though the rest of NIH grew by 2.6 percent. So there are ways for us to use both disease-specific planning.

The second example I can give you is the neurological institutes have now come together and are in the process of developing a blueprint for neurosciences, which is another mechanism where a cluster of like institutes can work together. And last is a trans-NIH process which we implemented over 1½ years ago called the NIH roadmap, which is a way for NIH to explicitly analyze where it is on the horizon of research and where it is that we need to make investments.

In this case we decided that the three areas would be molecular understanding of biological systems, new research teams and reengineering the clinical research enterprise.

So what are the next steps from my point of view that I could share with the committee in terms of how do we improve these processes that are there, that are operating? Can we make them better? Obviously we can always make them better, and we welcome all of the work that the committee is doing and that we are doing in trying to improve the processes as much as possible.

So how can we improve? One is we think—and all the directors agree—that we need to create better information systems to analyze the NIH portfolio of research. In one word, I think NIH has world-class peer review. We can make some advances if we invested in the information systems needed to analyze not just each grant but the totality of the grant portfolio both within institutes and across institutes.

The second recommendation would be that, just like the roadmap which I think worked well, we need to institutionalize a more regular process of trans-NIH priority review and planning with a common pool of funds.

The issue I think everyone raises is that the rigidity of the funding mechanisms do not allow institute directors, as well as the NIH director, to jointly and aggressively fund emerging areas of research. The fact that we have come together for the roadmap shows it is possible. I think this is a process; from my standpoint, I would increase tremendously the effectiveness of the agency. But it has to also come with a much more integrated governance and management system that includes all directors. We have started to do this with a transformation of how decisions are made at NIH.

And, obviously, we need to measure outcomes and have better measures. So I wanted to quickly go over the rationale that we fol-

low, Mr. Chairman, and I would like to have my colleagues continue the presentation. Thank you.

[The prepared statement of Elias A. Zerhouni follows:]

PREPARED STATEMENT OF ELIAS A. ZERHOUNI, DIRECTOR, NATIONAL INSTITUTES OF HEALTH, U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES

Mr. Chairman, Members of the Subcommittee, I am Dr. Elias Zerhouni, the Director of the National Institutes of Health. I am pleased to appear before you today to provide an overview of the NIH research portfolio and discuss priority setting.

NIH's mission is to conduct research that will lead to better methods of diagnosing, treating, preventing, and curing disease. The research that we support has resulted in improvements in detecting disease, better therapies, and more effective vaccines.

As one example, our ability to fight infectious diseases is greater than ever before. Consider how research advances enabled the world to quickly identify and contain the SARS virus. We will produce treatments and vaccines for other diseases, such as West Nile Virus, in record time. We have also developed a single dose, fast-acting experimental vaccine to prevent one of the most feared viruses of all, Ebola. The vaccine has proven successful in animals and human trials are under way.

In the area of mental illness, NIH-supported researchers recently discovered genes associated with schizophrenia, a tragic illness that affects 1 percent of the adult population. This research, which brings us closer to better treatments for this disorder, was cited by Science Magazine as the number two scientific "breakthrough of the year" in 2003.

Much of our progress is attributable to basic research advances furthering the understanding of human biology. NIH and its collaborators have sequenced the human genome, one of the greatest scientific achievements in history. Now we are moving forward with research into molecules and proteins to gain knowledge leading to new therapies that will alter the way medicine is practiced and result in even greater improvements in public health. We are on the cusp of an era of medical practice that will identify and prevent diseases before the symptoms appear.

In short, we are living much longer and significantly better as a result of biomedical research. And while we have come very far, we have even farther to go. Despite our extraordinary successes, there is still a great deal we do not know about human biology. In areas where we have reduced death and suffering, we can do even more. Consider the case of cardiovascular disease. One of the greatest public health success stories of the last half-century is the dramatic reduction in mortality from stroke and heart disease. In the year 2000, the number of deaths from cardiovascular disease was nearly 40 percent less than we had projected in the same year. Research identifying risk factors and new therapeutic interventions to control the risks were largely responsible for this remarkable lifesaving achievement. Yet cardiovascular disease still accounts for about 38 percent of all deaths in the United States every year. If we sustain our research effort, imagine how many more lives we can save over the next 50 years.

The Nation remains committed to the support of biomedical research. Congress and the President appropriated a little more than $28 billion to NIH in FY 2004, and the President's Budget Request for FY 2005 is $28.7 billion, a $729 million or 2.6 percent increase. Of the funds appropriated in FY 2004, an estimated $5.6 billion will be spent on cancer research and $4.9 billion on neurosciences research. We expect to spend $3.6 billion on research affecting women's health and $3.2 billion on pediatric research. We plan to spend $1.6 billion on biodefense research, and $2.4 billion on cardiovascular research. Another major investment is aging research, which will receive $2.3 billion. Vaccine development research will total $1.4 billion.

There are several ways to view the NIH budget. The most relevant picture is the snapshot of the individual Institute and Center budgets because Congress appropriates funds on the basis of allocations to these 27 organizations. In FY 2004, their budgets range from $4.7 billion for the National Cancer Institute to $65 million for the Fogarty International Center.

Another common way to view NIH's budget is by the funding "mechanisms", such as grants, contracts, cooperative agreements, or in-house programs. About 80 percent of the NIH budget is awarded to extramural research institutions throughout the United States. The largest grant mechanism is our Research Project Grants, which comprise 54 percent of NIH's budget in FY2004, or $15.1 billion. Another important funding mechanism, research centers, supports groups of investigators working on common disease or research areas. This mechanism accounts for 9 percent in FY 2004, or $2.6 billion. Another vital mechanism is our research training

programs, which comprise 3 percent of our total budget, and will help ensure that we will have the skilled workforce needed in the future to continue making progress in research. Our intramural research program accounts for 10 percent of our total budget, or $2.7 billion.

Our budget can also be viewed by funding of specific diseases, several of which I have already mentioned. Research projects can often contribute to advances in multiple diseases; thus, our estimates of research expenditures by disease necessarily contain overlap and are not mutually exclusive.

The public can also view the NIH budget from the perspective of the success rate of grant applications. However, looking at the budget from this perspective results in many misleading conclusions. Success rate alone is not indicative of the number or size of grants being funded; the number or quality of grant applications received in a given year; and research mechanisms that NIH may be funding other than grants. In addition, our estimates of the projected number of applications submitted and the number actually awarded have undergone significant revisions from earlier predictions, creating some difficulties in making grant budget projections.

Over the decades, the allocation of NIH dollars has adapted to public health needs. In fact, much of our spending focus recognizes the shift in disease burdens that has taken place in recent decades. For example:

NIH is increasingly targeting chronic diseases, which have overtaken acute conditions as the Nation's leading health problem.

We are responding to a new epidemic—obesity—which, if continued unabated, threatens to undermine our progress against disease in similar ways to tobacco use. Part of this response has been a proposed trans-NIH funding increase of 10 percent allocated for obesity research in the FY 2005 President's Budget.

We are quickly expanding our research efforts to protect the Nation against lethal bioterrorist acts by identifying the threats and developing vaccines, diagnostics, and therapeutics to address them.

We are committed to NIH's infectious disease research on problems such as AIDS, SARS, West Nile Virus, influenza, malaria and tuberculosis.

We remain committed to research on other long-standing problems, such as the health disparities that exist among racial, ethnic, and disadvantaged populations.

As the most influential force in the U.S. biomedical research community, NIH exercises its leadership by continually surveying public health needs and the scientific landscape to identify new biomedical research areas that require attention. Simultaneously, we search for emerging scientific opportunities. To maintain the vibrancy of our scientific enterprise, NIH also actively supports strong basic and clinical research training programs. Our programs are unique in both igniting and complementing private sector research and development efforts.

NIH undertakes studies for which the risks are too high or the financial incentives too low to attract private investment. Tailoring therapies for the special needs of vulnerable populations and evaluating treatments for rare diseases are other NIH-led investigations where the intervention of a public agency is essential. With the massive responsibility of advancing knowledge across such a wide landscape, whenever possible NIH marshals efforts of academic institutions, industry, research organizations, disease foundations, and patient groups to maximize its efforts.

This focus on vulnerable populations and rare diseases is an essential part of NIH's mission, and must be a component of priority setting. Instilled in all of us at NIH is the human dimension that drives us to help the helpless, whether their suffering is from a disease that affects millions or a disease that affects only a few. This is why I became a physician, and it is why I was eager to come to NIH.

To maintain a research portfolio that balances public health needs and scientific opportunities, NIH seeks input through multiple channels, including the Advisory Committee to the Director and the NIH Council of Public Representatives. NIH uses an unparalleled peer review system involving its Center for Scientific Review as well as separate vetting programs within each Institute and Center. These programs are part of a two-tiered system of advisory bodies and specialized review committees that guarantees funding of the best applications from among the nearly 50,000 research and training applications reviewed annually.

NIH's priorities are driven, in part, by the ideas and opportunities presented to us through the grant applications we receive. By placing most of our resources in investigator-driven research, NIH ensures that federal dollars track the latest science. But allowing the scientific community to drive research is only one factor in how NIH sets priorities.

Determining research priorities is a complex, multifaceted process. One cannot easily quantify the various factors and questions that surround priority setting at NIH. Some of the variables in the determination of resource allocations include pub-

lic health needs and the burden of disease, scientific opportunities, the quality of research proposals, the experience of applicants, and the ability to sustain research through adequate staffing and infrastructure. These factors are often lost in the public debate about NIH funding, in which the discussion is simplified by focusing attention on apparent funding inequities between the toll of certain diseases and the amount spent on research about those diseases.

Although burden of disease should not stand alone as a factor in setting priorities, there are indications that NIH funding generally tracks disease burden data. A study published in the New England Journal of Medicine five years ago concluded that there is a significant—although not absolute—correlation between the burden of disease and NIH funding. The genome project, development of instrumentation, training in clinical research, and new developments in basic science all have high values in the treatment of specific diseases, even though they lack a disease-specific orientation. Nonetheless, the study is evidence that NIH resources reflect the burden of disease in measurable terms.

Do these successes mean we are doing everything we can to ensure that the NIH research portfolio is balanced, is focused on the most urgent needs, and is based on irrefutable data? Let me answer that question with the following observation: Great organizations can maintain greatness only by continuous reassessment and adaptation.

I believe that we cannot be static. NIH must enhance the current process for determining priorities and allocating resources as part of a balanced research portfolio across the Agency and within each Institute and Center. The system of funding research by allocating resources directly to disease, organ, or special-population-based Institutes and Centers has served NIH and the public well. We plan to continue this approach to funding programs at the Institute and Center level. But science is changing, driven by new technologies and discoveries. Modern research is often best conducted by teams, which may include mathematicians, chemists, physicists, engineers, bioimagers, computer scientists, behavioral scientists, and physicians, and which may cut across the expertise of many different NIH Institutes and Centers. Several fertile areas of research—genomics, proteomics, molecular engineering—serve all fields of endeavor and cannot be pigeonholed according to specific diseases.

As the Institute of Medicine noted last year in its review of the structure of NIH, consideration should be given to refinements in the priority setting process and the management of our portfolio. There is a particular need for new and sustained approaches to evaluating NIH's crosscutting science. While maintaining the support for existing Institute and Center research programs, I think we should consider ways of using resources that may not be controlled by a single Institute or Center, but by a priority-setting process with input from outside and inside NIH. I am encouraging each Institute and Center to evaluate their own priority setting and portfolio management processes and seek best practices or other methods of enhancing their systems. I have also asked the Institute and Center directors to strive to pool resources, as they have done in research areas such as obesity and neuroscience.

An expanded approach to priority setting would enable NIH to ensure balance in our research portfolio, identify appropriate cycles of change, maintain proper turnover rates for grants and provide much more accountability to Congress and the public. Under such processes, we would identify crosscutting research that requires common investments from the various NIH Institutes and Centers. This approach must include a regular horizon scan of all research so that we can have sufficient information to manage the NIH research portfolio.

Two years ago, soon after I arrived as the Director of NIH, we convened a summit of the Nation's scientific experts to determine obstacles to the advancement of research and methods to overcome them that could not be addressed by any single Institute or Center. Teams comprising the NIH's leadership, working with their counterparts in the extramural scientific community, discussed new ideas. From these deliberations, the NIH Roadmap emerged. The Roadmap is focused on three goals: Identifying new pathways of discovery; Building the research teams of the future; and Re-engineering the Clinical Research Enterprise. As a modest but significant step forward, the Roadmap is supported by voluntary funding from all of our Institutes and Centers, with the goal of supporting research that will benefit all NIH programs and research into multiple diseases.

As I said, the Roadmap is a modest attempt at progress. It has an initial investment of less than 1 percent of NIH's total budget. The Roadmap is an example of a better-integrated mechanism for priority setting at NIH. My expectation is that we will build on the Roadmap, and it will serve as a model for future determinations of resource allocations.

In summary, I believe the confidence of the American people in NIH to lead biomedical research has been and will continue to be deserved. Our processes for iden-

tifying priorities and ensuring sound science have worked well. But reassessment and adaptation should occur and lead to a priority setting process that has greater public input, is more transparent, and lead to a research portfolio that will keep NIH at the leading edge of biomedical research.

I intend for the process to contain the following essential elements:

A transparent process characterized by a defined scope of review with broad input from the scientific community and the public.

A solid database of information, including uniform disease coding and accurate, current and comprehensive information on burden of disease.

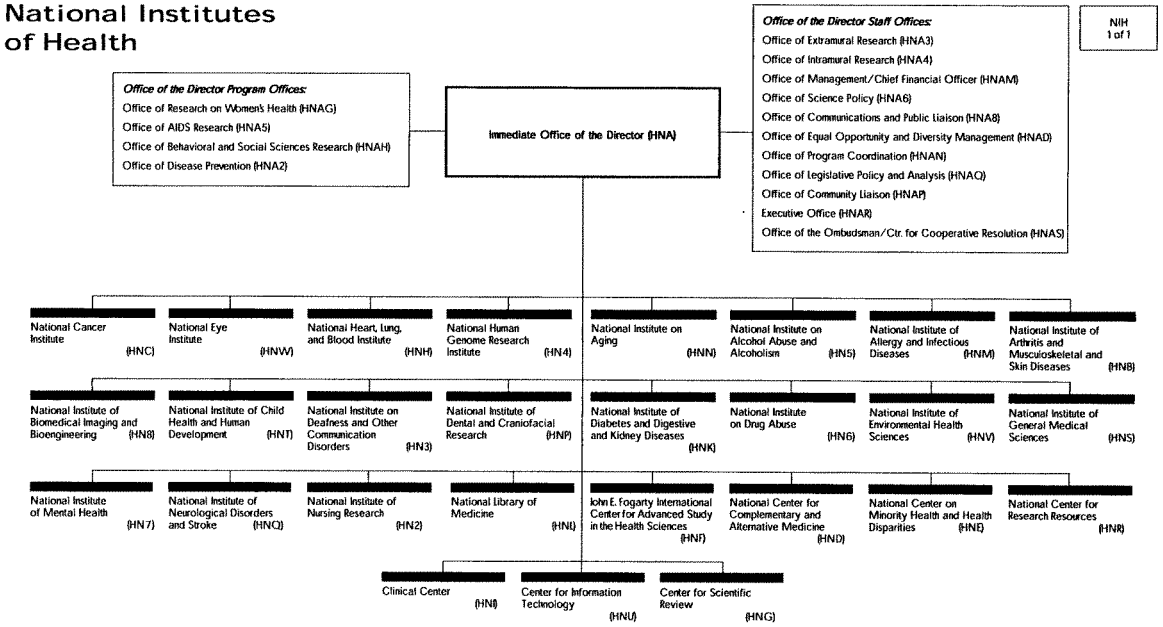
An institutionalized process of regularly scheduled evaluations based on current best practices to be used by Institutes and Centers.

The ability to weigh scientific opportunity against public health urgency.

A method of assessing outcomes to enhance accountability.

Thank you for the opportunity to testify. I will be pleased to answer your questions.

National Institutes of Health



The Mission of the National Institutes of Health is science in pursuit of knowledge to improve human health. This means pursuing science to expand fundamental knowledge about the nature and behavior of living systems; to apply that knowledge to extend the health of human lives; and to reduce the burdens resulting from disease and disability.

The National Institutes of Health seeks to accomplish its mission by:

- Fostering fundamental discoveries, innovative research, and their applications in order to advance the Nation's capacity to protect and improve health;
- Developing, maintaining, and renewing the human and physical resources that are vital to ensure the Nation's capability to prevent disease, improve health, and enhance quality of life;
- Expanding the knowledge base in biomedical, behavioral, and associated sciences order to enhance America's economic well-being and ensure a continued high return on the public investment in research; and
- Exemplifying and promoting the highest level of scientific integrity, public accountability, and social responsibility in the conduct of science.

NATIONAL INSTITUTES OF HEALTH
History of Congressional Appropriations, 1995 - 2004
(Dollars in thousands)

FISCAL YEAR	NCI	NHLBI	NIDCR	NIDDK	NINDS	NIAID	NIGMS	NICHD	NEI	NIEHS	NIA	NIAMS	NIDCD	NIMH
1995	1,913,819	1,257,374	162,430	726,949	627,045	535,199	876,778	512,165	291,095	266,566	431,991	228,176	166,660	542,200
1996	2,254,951	1,351,440	183,478	768,530	683,553	1,171,168	944,466	593,104	313,109	287,614	453,520	244,058	175,912	659,959
1997	2,389,065	1,431,830	197,063	813,149	729,259	1,257,794	995,471	631,628	331,606	307,562	484,326	256,228	188,273	700,701
1998	2,547,314	1,531,061	209,415	900,860	780,713	1,351,655	1,065,947	674,766	355,691	330,108	519,279	274,760	200,695	750,241
1999	2,925,247	1,792,509	234,183	1,020,559	902,680	1,569,063	1,197,026	750,485	395,595	375,494	596,126	307,960	229,735	860,638
2000	3,314,554	2,029,424	268,811	1,168,476	1,029,376	1,778,038	1,354,420	858,291	450,300	442,449	686,479	349,968	263,771	973,146
2001	3,754,456	2,298,512	306,211	1,399,684	1,175,854	2,041,698	1,535,378	975,766	510,352	564,810	785,590	396,460	300,418	1,106,305
2002	4,181,233	2,572,667	342,664	1,562,144	1,326,666	2,342,313	1,724,799	1,111,674	580,713	645,422	892,267	448,248	341,675	1,246,640
2003	4,592,348	2,793,733	371,636	1,722,730	1,456,476	3,606,789	1,847,000	1,205,927	633,148	697,767	993,598	486,143	370,382	1,341,014
2004	4,739,255	2,878,691	383,282	1,821,803	1,501,207	4,155,447	1,904,838	1,242,361	653,052	710,701	1,024,754	501,066	382,053	1,381,774

FISCAL YEAR	NIDA	NIAAA	NINR	NHGRI	NIBIB	NCRR	NCCAM	NCMHD	FIC	NLM	OD	B&F	OAR	TOTAL	
1995	290,029	181,150	48,164	151,518	..	284,693	..	..	14,646	125,303	217,882	114,120	$1,333,570	11,299,522	1/
1996	458,246	198,593	55,676	169,311	..	389,429	..	..	25,249	140,563	259,482	146,151	..	11,927,562	2/
1997	490,113	211,254	59,554	188,957	..	414,049	..	..	26,504	150,376	286,081	200,000	..	12,740,843	3/
1998	527,175	227,175	63,597	217,704	..	453,883	..	..	28,289	161,185	296,373	206,957	..	13,674,843	4/
1999	602,874	259,575	69,788	264,707	..	554,446	..	..	35,402	181,189	306,356	197,519	..	15,629,156	5/
2000	685,781	292,369	89,522	335,527	..	676,557	$68,390	..	43,494	214,068	282,000	165,376	..	17,820,587	6/
2001	780,833	340,453	104,328	382,112	..	817,253	89,138	$130,096	50,482	246,351	211,800	153,790	..	20,458,130	7/
2002	886,718	383,615	120,366	428,758	$111,861	1,011,262	104,451	157,563	56,859	276,091	235,113	204,600	..	23,296,382	8/
2003	961,721	416,051	130,584	464,995	278,279	1,138,821	113,407	185,714	63,465	300,135	266,232	628,687	..	27,066,782	9/
2004	990,953	428,669	134,724	479,073	287,129	1,179,058	116,978	191,471	65,382	317,315	327,504	88,972	..	27,887,512	10/

1/ Reflects enacted reductions of $7,446,000 for procurement, $345,000 for rent, $4,401,000 for bonus pay and a rescission of $20,533,000.

2/ Funds for HIV research in the amount of $1,410,925,000 appropriated to the ICs. Incorporates the NIH share of the government-wide administrative cost rescission ($5,780,000) and the Labor/HHS/E bonus pay rescission ($5,659,000).

3/ Funds for HIV research in the amount of $1,501,073,000 appropriated to the ICs. Incorporates the NIH share of the salaries and expenses reduction ($6,140,000) and the public/legislative affairs reduction ($220,000) National Center for Human Genome Research elevated to institute status in 1997.

4/ Funds for HIV research in the amount of $1,607,053,000 appropriated to the ICs. Beginning in FY 1998, includes funds appropriated to NIDDK for Type 1 diabetes research.

5/ Funds for HIV research in the amount of $1,800,046,000 appropriated to the ICs. Reflects rescission of $10,230,000.

6/ Funds for HIV research in the amount of $2,024,956 appropriated to the ICs. Reflects NIH share of across-the-board reduction ($99,883,000) and transfer to CDC ($20,000,000). Includes $40,000,000 in forward funding appropriated in FY 1999.

7/ Funds for HIV research in the amount of $2,244,987,000 appropriated to the ICs. Reflects NIH share of across-the-board reduction ($8,666,000) and transfer to DHHS ($5,800,000). Beginning in FY 2001, VA/HUD began appropriating Superfund Research funds directly to NIEHS.

8/ Funds for HIV research in the amount of $2,535,672,000 appropriated to the ICs. Reflects NIH share of across-the-board reduction ($9,273,000), Labor/HHS ($22,946,000) and government-wide ($34,243,000) rescissions, and transfer of $100M to the Global Fund for HIV/AIDS, malaria, and tuberculosis.

9/ Funds for HIV research in the amount of $2,747,483,000 appropriated to the ICs. Reflects NIH share of across-the-board reduction ($177,085,000), and transfers of $99,350,000 to the Global Fund for HIV/AIDS, malaria, and tuberculosis, and $583,000 to the Department of Homeland Security.

10/ Funds for HIV research in the amount of $2,850,581,000 appropriated to the ICs. Reflects NIH share of across-the-board reduction ($165,459,000), Labor/HHS rescission ($17,492,000), and transfer of $149,115,000 to the Global Fund for HIV/AIDS, malaria, and tuberculosis.

Mr. BILIRAKIS. Thank you very much, Doctor. Your general but very well-done presentation, I think, sets up all the other three, so I have allowed you to go well over your 10 minutes' time.

Dr. Fauci, please proceed with your statement.

STATEMENT OF ANTHONY S. FAUCI

Mr. FAUCI. Thank you very much, Mr. Chairman, and thank you for giving me the opportunity to discuss with this committee—and thank you, committee members, also—the priority setting process of an individual institute that in some respects represents all of the institutes but in many respects is unique because of the mandate of our mission to respond to emerging threats of infectious diseases.

On this first poster, as you see on your right, the mandate for the NIAID, the National Institute of Allergy and Infectious Diseases, as Dr. Zerhouni pointed out, the second largest institute with a budget of about $4.4, $4.5 billion, is research in immunology, microbiology and infectious diseases.

Now, if you look at the red arrows, these are the areas that we are responsible for. We are responsible for diseases of the immune system, infectious diseases in general, and then there are other issues that are thrown upon us by events. For example, the HIV-AIDS epidemic, emerging microbes, such as the threat of a pandemic flu and, most recently, biodefense. We set our priorities, just as Dr. Zerhouni mentioned, by the delicate balance that we continue to fine-tune because it is a dynamic process involving the scientific opportunities as well as the public health needs.

I would like to point out that both of those can change dramatically, because sometimes scientific breakthroughs create opportunities that just a year or 2 ago we didn't have, and sometimes public health needs like SARS comes along that you had no way of predicting.

So let me show you the history, the funding history of this institute, and how it has dramatically grown over the last several years. Not only because of the doubling of the NIH budget—because the scientific opportunities in immunology and infectious diseases are rather dramatic—but also as I mentioned, the HIV epidemic, the emerging and reemerging diseases like SARS, and then finally the rather dramatic increase that we experienced with the biodefense responsibility that has been given to us to develop countermeasures.

Let me just show you the dramatic metamorphosis of an institute that had to take place in the context of very serious continuing priority setting. Let me go back now to 1980 when things were, as we would say, stable. We had 60 percent of what we did with infectious diseases, about 40 percent in immunology, and that includes transplantation, asthma, and others. The budget, looking at our budget now, was relatively small, about $215 million.

Then let's fast forward to what we essentially say is the middle of the HIV epidemic. You know, we started to get significant funding right at the beginning of my directorship in the early 1980's. But let's take 1999, where AIDS, just as Dr. Zerhouni said, was something that was out of control. It isn't necessarily the numbers that we have now but the numbers that might occur. So it grew in a way that was in many respects disproportional if it crowded

out other areas; but because of the increases in funding, the other areas, which we refer to as "non-AIDS," also grew. But if you will look at that, more than half of the institute then was AIDS, half non-AIDS.

September 11, 2001, the anthrax attack, and biodefense, and here's what you have now; the institute in 2004, the current fiscal year, where it is about roughly one-third each of biodefense, AIDS and non-AIDS, non-biodefense.

Let me just spend a moment about emerging and reemerging infections. This is a slide that is a favorite of mine because I show it at many congressional hearings, as I am doing today, and the reason I like this slide is because I change it each year with one, sometimes two, and sometimes three additions. And what it tells us is the dynamic nature of how we need to prioritize. And, again, I gave you some examples. HIV-AIDS; West Nile in New York, when that was well off our radar screen many years ago; the ever-present threat of a pandemic flu that we had to address this year with the cases in Vietnam and in Thailand of bird flu jumping from a bird to a human. If it developed the capability of going human to human, we would have had to move very quickly. And in fact, we did. We made that prioritization right in the middle of a fiscal year.

Next.

So what is this prioritization process that I talked about? Well, as alluded to by Dr. Zerhouni, it is, again, a very dynamic process with input, from scientists particularly, to help us with understanding the scientific opportunity, but also involving lay public, the administration as well as the Congress, which is very sensitive—and we respect that—to many of the needs of the constituency. So we take that into account.

We have a number of processes. We have meetings. We have continual back-and-forth with blue ribbon panels. We have our council. But what I instituted about 15-plus years ago was two annual retreats, one a program retreat and one a policy retreat, in which we continually reevaluate not only the new initiatives—and I think this is important—but we do what Dr. Zerhouni referred to as a programmatical portfolio review, so that we don't get locked into something that is just getting funded because we have traditionally funded that.

That ultimately leads to the priority setting and the strategic planning and then the initiatives. What are initiatives? Initiatives are requests for applications or steering the field in a certain direction. We had to jump-start biodefense. There weren't a lot of people out there that were just dying to get into biodefense research. We had to cultivate the infrastructure, both human capital as well as physical.

This is the fruits of that process, and this is available on our Web site, but let me just point it out to you because I think it is an example of how we have been able to move quite rapidly. In the process of understanding that we were going to assume the responsibility for biodefense, we put together a strategic plan with input from many, many of our scientific colleagues, not only NIH-funded colleagues, but colleagues in the military and colleagues in other arenas.

We then developed the strategic plan-related research agenda for the Category A and the Category B agents, and then last summer we published our first progress report, and just a couple of months ago another progress report; together they encompass the Category A and B and C agents. Again, to underscore what Dr. Zerhouni said, this required our building the kind of infrastructure that years from now will allow us to make use of the scientific opportunities, but also to create scientific opportunities, which again, is a dynamic process that we continue to fine-tune.

Finally, your staff had asked me to address an issue that I briefed them on when they came to visit us at the NIH, and I want to spend the last minute or so on that. And that is again an example of the spectrum of going from fundamental basic research to the expanded paradigm for the NIH, namely assuring that we don't have a dead end with a very interesting observation that doesn't get translated into something for the public.

So if you look at basic research on the far left and look at where we want to go, and that is, we want countermeasures for biodefense, we want diagnostics, therapeutics, we want vaccines, we want the same thing for HIV-AIDS, and we want it for things like SARS.

We must take the initiative, and we are doing that now with our prioritization, and pushing the process more toward the preadvanced development so that we can meet industry halfway or beyond.

On the one hand, we have created incentives. Let's take biodefense as an example, with Project BioShield, with a secure source of funding to buy products that we engage very heavily in the concept development and basic research. Now we find in our analysis of our budget—and we have had intensive discussions with Dr. Zerhouni on this—now that the pipeline for many things that we invested in basic research are getting robust, we need to push that process forward. So we need to reprioritize now the balance between the fundamental basic research portfolio and how we push that to development so that we wind up meeting the needs of the general public which have put in our trust the money for the research that we are doing.

So this is a process that I think is an example of how the NIH in a dynamic way continues to evolve to meet the challenges, be it challenges of fundamental diseases that have been around for a very long time, or the unexpected, like SARS, like AIDS, like biodefense.

So I will end my comments there, Mr. Chairman, and be happy later to answer any questions. Thank you.

Mr. BILIRAKIS. Thank you so much, Doctor. Fascinating.

Dr. von Eschenbach.

STATEMENT OF ANDREW C. VON ESCHENBACH

Mr. VON ESCHENBACH. Thank you, Mr. Chairman and distinguished members. It really is not only a great privilege for me, but an important opportunity to address this prestigious committee on scientific opportunities and public needs and balancing those priorities.

As we sit here today, one American every minute is dying of cancer. More than a half a million people will die from this disease this year, and more than 75 percent of families are affected. One in two men and one in three women will be told during their lifetime that they have cancer.

Congress recognized the horror of cancer and the need to make the conquest of cancer a national priority in 1971 when it passed the National Cancer Act, which authorized the NCI Director to build and lead our Nation's cancer program. Thanks to the wisdom of Congress and your continued support, I am pleased to follow up on the efforts of my predecessors and report to you that scientific progress is now impacting on the greatest public health concern of the American people, the fear and problem of cancer.

During the past decade, for the first time ever, we have seen mortality rates from cancer decline. This has been especially true for the most prevalent cancers, lung, breast, prostate, colon and rectal. In 1971, there were only 3 million cancer survivors alive in the United States. As Dr. Zerhouni indicated, that number today is almost 10 million. But the greatest progress is yet to come.

Progress in cancer research that has been made possible by the authorizations of the National Cancer Act of 1971 and the continued appropriations provided by Congress has really created an opportunity both scientifically as well as with regard to the delivery of care that is really at this point transformational. It is now making it possible for us to envision a future in which no one will suffer and die as a result of cancer.

The National Cancer Institute is committed to continuing to fulfill the promise of bringing that reality about. We can do that now, and we have established a goal of eliminating the suffering and death due to cancer and making that a reality by 2015 because we now are beginning to understand cancer as a disease process. We now recognize that there are steps at the genetic, molecular and cellular level that are responsible for our susceptibility to cancer, that are responsible for the early premalignant changes that occur, and then those processes continue to result in the overt development of a tumor, and then that tumor's growth and dissemination and spread until ultimately it takes a patient's life. Progress in biomedical research that has come about because of the effort of the National Cancer Institute in leading our national cancer program is not only unraveling the steps in this process, it is also providing the insights into the development of interventions that can preempt this process. We now can envision prevention, detection, elimination and modulation of cancer in a way that people will either not develop cancer in the first place; if they do develop cancer, we can detect the disease early and eliminate it much more safely; or we are able to treat and modulate established cancers such that people will live with but not die from cancer.

In order to bring this goal about, in order to establish the priorities and the investments that are necessary to achieve this goal, we have created a priority-setting process and a planning process that really defines, if you will, a balanced portfolio, a portfolio of initiatives that are involved in discovery, initiatives involved in development, and those that are involved in delivery. Across the entire portfolio of the National Cancer Institute, there are strategic

priorities and initiatives in all of these areas such that through the process of discovery, development and delivery, we will create those opportunities and deliver those opportunities to patients in need to achieve that goal of eliminating the suffering and death due to cancer.

In the process of establishing the portfolio, we engage in a very elaborate and continuous process of providing and obtaining input into the establishment of priorities and in processes that review those priorities prior to implementation, and then also processes that determine the impact of those priorities. In order to give you an insight into that, I would like to just lead you through how that ongoing planning and budgeting process occurs.

It begins with a constant set of opportunities for input into the establishment of those priorities. Those inputs come from a variety of places and from a variety of organizations. We have mechanisms in place that help us develop our annual planning document and budget document that we call the bypass budget. There are a series of targeted advisory groups and disease focus groups that provide specific input, one of which has been the ongoing series of the progress review groups which has looked at opportunities and needs in areas like breast cancer, prostate cancer and others. We have efforts that are under way with regard to state-of-the-science meetings in which we can look at some of those emerging scientific opportunities that Dr. Fauci and Dr. Zerhouni alluded to, and we make certain that we have significant input from the world community, especially cancer survivor groups and organizations like the American Cancer Society.

All of that input is then synthesized into an internal planning document that then becomes the basis of both our strategic plan as well as our business plan or budget. That internal document is prepared by senior NCI leadership with broad input from the entire NCI and is processed by our executive committee. That is then reviewed on an ongoing basis by initiative and by priority by formally chartered advisory committees including our National Cancer Advisory Board, our Board of Scientific Advisers and our Board of Scientific Counselors as well as individual groups of expertise.

Once the programs have been vetted, they are then implemented and approved by passage through the National Cancer Advisory Board, which serves as our council, and then disseminated and implemented throughout the entire cancer community. With the implementation of those programs, we have the opportunity for continued monitoring and surveillance that gives us opportunities to determine measured outcomes and results, which then feed right back into the ongoing planning process.

It is through this decisionmaking process that is focused on making strategic decisions about the balance in our portfolio and making sure that that portfolio is constantly being directed toward our mission that is the process by which we establish and set priorities and balance the scientific opportunities with the public need. Our ability to disseminate that information through the professional judgment budget or the bypass budget on an annual basis provides opportunities for insight into both the strategic plan as well as the budget or business plan that is required. This is not, however, our budget submission process. That occurs directly through the mech-

anisms that are available within the NIH and directly to the Director of the NIH and then on to the Department of Health and Human Services.

In addition to these formal processes, we have also looked at opportunities to significantly increase our communications with the community and have recently launched a weekly cancer bulletin that is available on the Web as a way of communicating to the entire community our scientific priorities and also the scientific achievements that those investments are bringing about. In doing so, we hope to continue to fulfill the mandate and mission of the Congress to conquer cancer and eliminate its suffering and death. Thank you, Mr. Chairman.

Mr. BILIRAKIS. Thank you very much, Doctor.

Dr. Volkow.

STATEMENT OF NORA D. VOLKOW

Ms. VOLKOW. Thank you very much, Mr. Chairman and members of the subcommittee. It is a privilege for me to be here and participate in this hearing.

I will not describe the process by which we set priorities because it is similar to those described by my colleagues. Instead I will share with you our research priorities and will highlight the unique collaborations that NIDA has had to cultivate in order to translate science to communities.

Like the other institutes, NIDA receives input about its research priorities from a wide variety of sources, including our National Advisory Council, scientific and health professionals, and policymakers. However, unlike many other medical diseases, addiction does not have many patient and family advocacy groups. This is in part due to the fact that drugs of abuse in most cases alienate the addicted person from his family and his community rather than eliciting support. This places additional importance on NIDA's ability to support science that helps us identify national needs and emerging priorities.

The disease burden attributed to drug addiction is enormous. It is estimated to cost for both legal and illegal drugs more than $484 billion a year. However, even as large as this number may seem, it pales in comparison to the devastating consequences of drug abuse to the individual and to society.

Drug addiction is a disease that targets the brain, modifying its function in ways that limit the individual's ability to make decisions on his or her behavior. The results are widespread and devastating and can include family disintegration, child abuse, loss of work and income, accidents, criminal behavior, mental illness and suicide. Moreover, because drug addiction develops during adolescence and even sometimes in childhood, it can shatter the life of an individual from its early beginnings. Drugs of abuse not only affect the brain, but many organs in our bodies, thus also contributing to the burden of many medical diseases including cancer; cardiovascular, pulmonary, and infectious disease; even obesity.

Research priorities at NIDA are set by the urgent need to decrease drug abuse and its consequences while at the same time taking advantage of scientific opportunities to increase our knowledge about addiction.

Prevention and treatment of drug abuse and addiction are NIDA's top priorities. Prevention is particularly relevant since adolescents and children are the most vulnerable victims for drugs of abuse. Moreover, research has shown that prevention works, and this is illustrated on this poster from a study that monitors, in teenagers, the perception of the harmful effects of drugs versus the prevalence of drug abuse. When students perceive drugs to be risky, their rate of drug abuse drops. In fact, we are finding that through our monitoring mechanisms, we can often predict the prevalence of drug utilization on the basis of the perception of drug risk detected the year prior.

Unprecedented scientific opportunities on prevention research have emerged from the identification of genes that affect the responses to drugs of abuse and also by the development of technologies that now allow us for the first time to evaluate the function of the human brain. We can now investigate questions that were heretofore inaccessible, such as how does early drug exposure affect the development of the human brain, such as what is the relative contribution of genes versus environment in drug addiction, such as how do environmental factors and genes affect our brain and how that in turn affects behavior.

In treatment our priorities include the development of medications that can counteract the effects of chronic drug utilization while at the same time developing research that optimizes our ability to bring the science into the community.

Another priority in treatment is addressing the medical consequences of drug abuse. Drug abuse is frequently comorbid with mental illnesses and with other medical diseases. In many instances this comorbidity results from the role that drugs of abuse have as a contributing factor on the medical illness. For example, drug abuse is one of the leading contributors to the spread of HIV/AIDS in our country, not only by injection drug use, which accounts for 36 percent of the new HIV cases, but also by drug intoxication, which interferes with the judgment of the person and increases the likelihood of risky sexual behavior. Thus, treatment of addiction and prevention will have an impact on the prevalence and the prognosis of other medical diseases.

Scientific opportunities on treatment research have also emerged from information derived out of the genome project, which has allowed us to identify a wide array of new compounds that in animal models interfere with drug administration. However, notwithstanding the series of very promising compounds, a major roadblock into their testing for clinical utility has been the limited involvement of the pharmaceutical industry on the development of medications. Issues such as stigma, lack of reimbursement for drug abuse treatment and the perception of a lack of a large enough market are some of the variables that make companies reluctant to get into the development of antiaddiction medications.

For science of prevention and treatment of drug abuse to have an impact, NIDA relies on its collaborations with other NIH institutes as well as its partnerships with other agencies and organizations to help bring this knowledge into the community. Indeed, the successful 11 percent reduction in teen drug use during the last 2 years reflects the power of several agencies working together to-

ward a common goal. These collaborations include not only the medical community such as pediatricians and general practitioners for early drug abuse detection, but also partnerships with agencies such as the Substance Abuse and Mental Health Services Administration, or SAMHSA, and the White House Office of National Drug Control Policy, or ONDCP. It also reaches to the Department of Education to bring prevention interventions into the school environment and the Department of Justice to bring treatment strategies that will minimize the chances of recidivism and reincarceration once inmates with drug abuse problems leave the jail or the prison system. We also work with State and local agencies to bring science into the communities.

Though we have made significant progress in our understanding of drug abuse and addiction, there is still much more we need to know. Fast advances in knowledge and technology provide us with opportunities to exponentially expand our understanding of how our brain works and how it molds behaviors. In the case of drug abuse where drugs directly affect brain function and where the environment can play either a permissive or protective role, new knowledge will help us develop more effective prevention and therapeutic strategies.

I will be happy now to answer any questions you may have.

Mr. BILIRAKIS. Thank you very much, Doctor.

Let me ask you, I believe it was Dr. Zerhouni who addressed obesity. Would the obesity research that is conducted be spread throughout more than one institute?

Mr. ZERHOUNI. It is.

Mr. BILIRAKIS. It is, right?

Mr. ZERHOUNI. It is. The trans-NIH Obesity Task Force is actually a multi-institute effort. It was led by Dr. Spiegel, the head of NIDDK, diabetes and digestive disease institute and heart and lung. The reason it is many is because it affects children, so NICHD is involved. It has an impact, obesity, on influencing the rates of cancer, so NCI is involved. There is obviously a component of neurobiology, so the Neurological Institute is involved. So it involves a large number.

Mr. BILIRAKIS. The reason I pick on it is sort of to help me to try to get the picture. You indicated—I think you said there was a 10 percent increase in obesity research funding as against an average of 2.5 percent increase or something like that. Did that 10 percent come about independently? In other words, these institutes determined how much money should go toward obesity research in these particular institutes so that the ultimate total increases of all those turns out to be 10 percent?

Mr. ZERHOUNI. Both ways. The total portfolio in 2003 was nearly $400 million of research. It grew from about $85 million 8 years ago to almost $400 million in fiscal year 2003 because of the burden of the disease. The second is that obesity has been declared an area of priority for all of medical research a while back.

So how are the nearly $400 million distributed? Fifty-five percent of that money on average, and I am not exactly accurate about obesity, but 55 percent will be distributed because scientists come to us with ideas about how to understand obesity better. This is what we call the investigator-initiated funding. About a third will come

from clinical trials that we are doing. For example, NIDDK conducted a trial in children with obesity comparing diet versus exercise in the appearance of diabetes in obese children. That was about a third of the expenditures. Those tend to come from what we call initiatives. So the institute, for example, NIDDK, issued what we call a request for application to have people come forward and conduct trials that we are interested in conducting.

As you can see, there are two components. There is a directed component of the portfolio. This is what we call initiatives that Dr. Fauci mentioned. And there is an undirected component which responds to scientific proposals that come to us. The 10 percent that we did by which we increased the portfolio came from this planning process which I insisted be done, asking the directors to come together and look at the obesity portfolio across whole institutes, and it was decided that there would be two priorities, two new priorities. One, we think it is very important to accelerate our research in obesity in childhood. All the evidence suggests that obesity is determined very early in life. We thought we didn't have enough investments in early childhood obesity, so we increased our investment there. The second is obesity really harms an individual not because of obesity itself, but because it increases the chances of cardiovascular disease and diabetes and other what we call comorbidities. So what we are thinking is that research needs to be done to disconnect very quickly as much as we can in the population obesity from the emergence of diabetes and other comorbidities.

Mr. BILIRAKIS. So there was—I think you used the word "we" a number of times.

Mr. ZERHOUNI. At the end of the year when the budget came, when we presented our budget to the Department, we set aside $40 million; $22 million of that $40 million was dedicated to these new areas that were deemed unserved at that point, the childhood obesity and the comorbidity research.

Mr. BILIRAKIS. Would we say then that "we" was your advisory council along with you that made those decisions? Did you make them in coordination with the 27 institutes and centers?

Mr. ZERHOUNI. That is correct. What we did since I became Director, we have reorganized the way we make decisions at NIH. We had 27 directors. It is very complicated to have that many, so we created a steering committee of nine directors, a smaller number that look over all the major corporate decisions that NIH has to make. The budget is decided obviously between the NIH Director and all of the directors that participated in this NIH initiative, the trans-NIH obesity.

But it is limited. Our ability to move dollars from one portfolio to another is limited. It is not something that you can do arbitrarily, because programs tend to go over for 3, 4 years, and they are committed for that period of time. We need to do it with the appropriate oversight. What I think needs to be more encouraged, and we are encouraging it, and the institute directors can comment, is more planning not within the institutes, which is done very well, in most cases it is the way to go, but planning across diseases that affect more institutes, and areas of research that af-

fect more institutes, and areas that affect all of NIH with dollars attached to it.

Mr. BILIRAKIS. My time has expired, but hopefully we can get back into that as time goes on.

Mr. Brown.

Mr. BROWN. Thank you, Mr. Chairman.

Dr. Zerhouni, would you briefly comment on my opening comments about Duchenne? They actually funded only one clinical trial. Just give us a fairly brief answer to that, if you would.

Mr. ZERHOUNI. Sure. First of all, I know that muscular dystrophy has increased in funding. The funding is about $40 million, so it is about 15 percent of the NIH budget. I understand that the MDCARE Act is the mechanism, the vehicle by which we are coordinating all of the portfolios of muscular dystrophy.

You mentioned the issue of centers. I understand that three were funded in 2003, and up to three will be funded, 2 to 3 will be funded in 2005.

I also can tell you that we have to be very careful when you ramp up research capacity, you have to make sure you have the people and the ideas there to make it happen. So review is very important. In the previous cycle, our review was indicating some reservations about the maturity of some of the centers. But by and large what I think needs to happen is more investments in a coordinated fashion in a set of centers that would focus on that aspect.

I don't have information about what you said about clinical trials and having three applications. I really can't comment. I would like to get the information and forward it on to you.

Mr. BROWN. Thank you.

Dr. Fauci, in Dr. Zerhouni's written testimony, he spoke, and I am quoting, of NIH undertaking, quote, studies for which the risks are too high or the financial incentive is too low to attract private investment. A lot of us on the subcommittee are concerned about a couple of things. One is that the lack of research or the inadequate research on infectious disease, especially in the developing world where it is hard to imagine it would be very profitable for a prescription drug company, a pharmaceutical company in this country; second, the emptiness, if you will, of the antibiotic pipeline.

Could you comment on this? How much of the NIH budget typically is devoted to that kind of research; how we can assist you to do better, especially in the area of antibiotics, with antibiotics, with antiparasitics, with antiretrovirals, and especially with drug resistance in much of our antibiotic, antiparasitic supply?

Mr. FAUCI. That is an excellent question, Mr. Brown. We take the responsibility in our emerging and reemerging diseases program to address issues such as antibiotic resistance. This is one of those areas where we absolutely need to deal on a closer basis with our industrial partners. That is the delicate balance that I was talking to you about, because they have incentives to get into areas that are high profit margins for them. That has to do not only with antibiotics, but also with vaccines.

What we have been trying to do, and that is the reason I showed that slide and why the committee staff wanted me to show it at

this particular hearing, was that we need to figure out ways—and I can't give you a list of one, two, three things that you can do vis-à-vis legislation or what have you, but I would be very happy to work with you and your committee staff to figure out ways how we might be able in a better way and in a more facile way to deal with our industrial partners so that we can get them interested in the things that we can do in the normal interaction that we have.

One of the steps forward was the bioshield initiative was very specific for biodefense. It created ways of dealing—in a much more streamlined way of dealing with the industrial partners, but also for giving them the incentive to get involved in something even though it was not a guaranteed profit margin for them. I think we need to look at that model as it applies to all emerging and re-emerging diseases, particularly diseases that we refer to as the neglected diseases.

We have a portfolio of research, but we need to get the companies involved. We cannot do it all ourselves. That is the reason why, as the months and years go by, we continue to interact with the companies, and we are doing it much more now than we have done years ago.

Mr. BROWN. What are the neglected diseases, TB, malaria, those that don't have much of a market in this country?

Mr. FAUCI. There are two types of neglected diseases, infectious diseases I am referring to now. There are those in which the burden of disease is extraordinary, but there is not necessarily a lot of research going on. Malaria and TB are the two big ones on that. Do you know that we have, for example, the vaccine for tuberculosis, BCG, which is quite ineffective in preventing the adult type of infectious tuberculosis that spreads from person to person, but is pretty effective in preventing meningitis complications in children. Yet we now on our own initiative—and this is one of the things that we talked about—when we looked at the portfolio, there wasn't a lot of action going on in TB vaccine research. So we seized the opportunity of the capability of the sequencing of microbes that we can do right now and the ability to use proteomics and postgenomic function to develop a vaccine that we are now testing in clinical trials which, believe it or not, it is amazing to say this, the first new tuberculosis vaccine trial in this country in 60 years, which we just started this year. We did it because we were able to translate the opportunities that we had with the new capabilities of sequencing the genomes of microbes with the new modern-day molecular biology. It isn't the old vaccine, based on the entire microbe, but a very small molecular component that we call a fusion protein that will allow us in a much safer way to do a vaccine trial for tuberculosis. If that is successful, I think we are going to be able to transform the entire landscape of tuberculosis.

Mr. BROWN. What is an optimistic assessment or estimate of how long from where you are now until it can be used in the worst TB places in the world like India?

Mr. FAUCI. When you talk vaccine development and ultimate approval, you are always talking several years, 8 to 10 years. I would imagine that if we accelerate the process, which we are doing right now, we might be able to shave a year or 2 off of that. But you are not talking next year or the year after. If you are talking about

full FDA approval, the kinds of things we need to do for safety, it is going to take several years. It is being tested now in our network of clinical trials.

Mr. BROWN. Thank you.

Mr. BILIRAKIS. Mr. Shimkus to inquire.

Mr. SHIMKUS. Thank you, Mr. Chairman. Mr. Greenwood was here way before I.

Mr. GREENWOOD. I am not sure that I was.

Mr. BILIRAKIS. That is not what the staff tells me.

Mr. SHIMKUS. Okay. I am ready. Thank you. Thank you, Mr. Chairman.

I have great respect for my friends Ms. Capps and Mr. Waxman and in their statements. They are noted health observers and professionals in the field. But speaking from a very conservative area of the country, we talked about this at the last bicameral hearing we had on the Senate side last year, that it helps us in rural America if the grants that are issued pass the common-sense test.

The question is, is there a way that you can through this evaluation process bring some sense or explanation on those that don't? We don't have to go through them. They have been publicly written about for years now. All of us get lobbied strongly in support of the research done. It was Speaker Gingrich who really pushed to double the size of NIH, and we have made great investments. When we are asked for more and more dollars in periods of tight dollars, we want to make sure that those dollars are best spent. So how do we address again—where is the common-sense application on some of the research dollars?

Dr. Zerhouni, if you would answer that first, and then I would probably like to follow up with Dr. Fauci.

Mr. ZERHOUNI. This is a very important question. We are very concerned. In fact, the Chairman mentioned the term transparency. I think in this area we found after our review that we could do a lot better in making sure that we communicate transparently and also fully about the importance or lack thereof of the particular research. So one of the things that I have done after reviewing this field, in conjunction with all of the directors in our extramural office, is to issue new requirements for explaining, in plain language, both the public relevance as well as the importance of the research scientifically. This information will be available in clearly understandable language both to the public and to the multiple review levels that we have in place so that there will be more transparency and more explicit understanding of all areas of research.

The common sense test that you rightly bring up is something that we are quite concerned about because we depend on the support of all taxpayers and we need to make sure that whatever we do makes scientific sense and public health sense. In that context I have asked all of the institute directors to make sure that the two level of reviews are done fully. I know it is a lot of work, but that there is a full discussion of the grants at the advisory council level, because there are public members in those mandated by law in these advisory councils, and I think they should play their role.

This is why I had this report made by the Council of Public Representatives called, "Enhancing Public Input and Transparency in the National Institutes of Health Priority-Setting Process" that ad-

dresses it, but I don't think that we can weaken the peer review process in trying to answer the concerns. We need to make sure that we accomplish both.

Mr. SHIMKUS. I applaud that. Somehow I would just hope that as we move to more transparency, that that helps and doesn't hinder. Again, as many of us would question the common sense of the application of some of these grants, more transparency may make it more difficult for us to defend the NIH.

Mr. ZERHOUNI. Although when I reviewed the grants, frankly, the language was highly scientific with terms of art that were not explained as well as they should be or could be. I think we should do better and then obviously review the question.

Mr. SHIMKUS. I did mention Dr. Fauci, but I guess any of the directors if they want to. It is up to you. My time is almost out, so if someone else wants to add, you may do that. The same area.

Mr. FAUCI. We do the same thing. Obviously in areas such as HIV/AIDS, it is a sexually transmitted disease, it is a disease that is transmitted by injection drug use, by a variety of other mechanisms. We cannot avoid addressing the issues that are at the very foundation of why millions and millions of people are getting infected. That is the reason why we are sensitive to the issues that you bring up, really quite sensitive, and I mean that sincerely. But we need to let the science drive the questions if we are going to be able to get a handle on this very devastating sexually transmitted disease.

Mr. BILIRAKIS. Mr. Strickland, you have 8 minutes.

Mr. STRICKLAND. Thank you very much.

Dr. Fauci, I have read and have been concerned for a number of years about what some say is the potential for a pandemic, an influenza pandemic, occurring across the world that could perhaps consume the lives of millions of people. I don't know if what I have read is just reason to be concerned or not, but the question I would like to ask you, is this a concern, is it a possibility, and if it is, do you feel like we are doing everything we can to be ready for such an occurrence?

Mr. FAUCI. It is a possibility because it has happened in 1918; to a lesser extent in 1957 and 1968. It is of concern.

In science and public health, it is very rare that we can say we are doing absolutely everything that can be done, but I can tell you, Mr. Strickland, that we have put this at the very highest priority. This is one of the things that I mentioned to you in midstream we had to make adjustments in our priority setting. For example, and I will be very brief on this, but it is important because you are interested in this, and it needs to be understood. There is what is called interpandemic influenza, which the NIH and the CDC and the FDA have been involved with for decades and decades, where you look at the burden, and you look at the particular microbe that is circulating, and you work together to have a vaccine for the next interpandemic flu. Each year—it is very unappreciated: 36,000 people a year die from plain old flu, 114,000 hospitalizations. It is a very serious disease. I think it suffers from the semantics of, oh, I have the flu, when you don't really have the flu. You probably have a relatively benign rhinovirus or something like that or a coronavirus.

What we are doing now in our preparation is that something different happened over the last few years that started in 1997 when a bird flu jumped from a bird to a human. By killing and culling the birds in Hong Kong, the lid was put on that. And then successfully over the next few years until this particular winter, 2003-2004, nine countries in Asia had the emergence of a virus among flocks. In two countries, in Thailand and in Vietnam, there was a total of 34 cases of which 23 died. That is nearly a 70 percent mortality. The concern we in the Department, particularly the NIH and the CDC, have is that that microbe has the ability now to jump from chicken to human. The reason it isn't a disaster is because it hasn't yet learned how to go from human to human. So the potential epidemic has kind of smoldered and stopped.

What we have been doing now is that we have been doing basic research as well as developing a seed virus vaccine that our grantees and contractors have developed. We have taken the responsibility, even though we had to do midstream corrections. This is something that I discussed in some detail with Dr. Zerhouni and got his encouragement to move ahead with it, and to now start making a pilot lot, which we are in the process of doing.

Again related to the question that Mr. Brown asked, we had to get very much involved in our industrial partners, in this case it was inventors Pasteur and Chiron, in developing a pilot lot and then to have that be able to scale up if necessary at commercial levels. So we are doing everything within the resources that we have right now because we put it as a very high priority.

It is an example—again, just to get back to what Dr. Zerhouni said a few minutes ago, the disease burden right now in the United States for pandemic flu is zero, yet we are putting resources into it, and we plan to do more next year because we know the potential for that is enormous. We are part of the whole Department. We have an HHS-based pandemic influenza plan that is led at the level of the Department that we, the NIH and the CDC and the FDA, are a very important part of.

Mr. STRICKLAND. Do you feel that the communication or that the data-gathering infrastructure around the world is sufficient to enable you to be alerted and to act as quickly as possible based on what you currently have in existence?

Mr. FAUCI. Yes and no. I will tell you what the yes is, and then I will explain the no. The yes is that we have a number of collaborating WHO centers of which the Department, namely the FDA and the NIH and the CDC, play an important role at. We have a grantee of ours who has a major program in Hong Kong. So when you talk about flu, almost invariably it is going to emerge from China, Hong Kong.

Mr. STRICKLAND. Because of their agricultural practices?

Mr. FAUCI. Because of the sociological and economic conditions there. You have pigs and ducks and chickens and people working on the farm together, a natural mixing bowl for a virus that would jump from one species to another. That is the yes. So we do have these people in communications. For example, when the bird flu came out, we immediately dispatched a person to Hong Kong to start working on it.

The no to maybe is that we have not had complete transparency up to now, but it is getting better and better with our Chinese colleagues. We saw that with SARS, which was recognized months and months before in China until we knew about it, and we only knew about it when it got to Hong Kong where we had our people on the ground. With the flu now it is getting better, but I don't have 100 percent confidence about the transparency yet. But it is certainly much better than it was before.

Mr. STRICKLAND. I want to thank you for your answer. If the potential consequences are so great, it is something that I think we certainly should put all the resources that are needed into it.

I would just like to say a word about the comment my good friend on the other side made about the common sense test. It seems to me that the common sense test is not relevant because it is common, and that which is easily or readily understood or appreciated is not, it seems to me, the major domain of the scientific inquiry. You want to look at that which is not common or easily or readily appreciated or understood. It seems to me that is what the scientific inquiry is all about.

I have appreciated you being here. I wish we could spend hours because there are so many issues. What you do, I think, is as important as anything that we consider in this committee or in this Congress, because you have cancer potentially being cured in 15 years. I mean, with all due respect, I asked my colleague if she thought you were maybe a little off to think of that. I am speaking facetiously and trying to be humorous here, I guess, but to think of that, it is overwhelming. How great it would be. And then I said to her, it would absolutely destroy our Social Security system. But what you do is so integral to everything else we consider in this Congress, economically, socially. We could talk forever about the social implications of the stem cell research policy or of the abstinence only education policy or the drug policy.

Mr. Chairman, I just wish we could do this more often and for a longer period of time.

Mr. BILIRAKIS. You have had more time than anyone else.

Mr. STRICKLAND. Thank you, sir. I appreciate it.

Mr. BILIRAKIS. Mr. Barton to inquire.

Chairman BARTON. Thank you. My questions are going to be more operational and structural and not going to be policy so much. But my first question to you, Dr. Zerhouni, if you were starting from scratch with a clean sheet of paper to create a National Institute of Health, would you come up with 27 institutes and centers?

Mr. ZERHOUNI. No.

Chairman BARTON. Is there a magic number?

Mr. ZERHOUNI. No. I think you really would like—if I had my magic wand, I think what you would want is an evolving structure that evolves easily and flexibly according to its priorities. History dictates a tremendous amount of the structure of NIH, history, congressional actions, legislation, which really creates a degree of rigidity, which, from my standpoint, needs to be thought through. A process is needed by which that structure needs to be reviewed at regular intervals to ask the obvious question, do we have structures that still fit the reality of today. The rigidity, sir, is something that I think would be a good topic of interaction.

Chairman BARTON. Under current law, do you as the Director have the authority to restructure, recombine institutes and centers, or are they set by law and you have to go with what is there right now?

Mr. ZERHOUNI. I would say it is almost completely limited. I can do some restructuring within the structure. That is very difficult to do. Remember, we have institutes and centers. In my office, for example, we have program offices. Of the about $290 million budget that you see within the Office of the Director, there are mandated offices with their own budgets: the Office of AIDS Research, Office of Behavioral Science, Office of Rare Diseases, of which I have very little to say in terms of programmatic spending. So at the end of the day, you end up with about $120 million all together that the Office of the Director directly controls.

But I think the lack of a process of adaptation, and to speak in terms of policy and long-term future, there is no such process that would allow a reasoned, learned evaluation of appropriateness of structure relative to mission.

Chairman BARTON. As we move toward reauthorization, would it be appropriate for the legislation in conjunction—working with the stakeholders to create a new structure, or would it be more appropriate to give the Director's office the ability to do the restructuring, the authority to do the restructuring?

Mr. ZERHOUNI. I think, in my view, since you have the reality in this institution, NIH is still a wonderful institution that performs very well in most aspects. I think what would be more important is the process, with authority, obviously, to look at certain structural elements and the need for them to change. But it should be mandated in some fashion.

Chairman BARTON. I want to give the center directors and institute directors a chance on that last question. You may not want to change the structure. You may think 27 is great. That is a fair policy position. But if you think there needs to be a reorganization, do you three ladies and gentlemen want us to provide it, or do you want us to in some way give the institution the authority to do it?

Mr. FAUCI. I think it would be fraught with danger, sir, if you legislate structural changes as opposed to providing the kinds of flexibility that would allow the NIH to evolve with the scientific evolution of things, the way Dr. Zerhouni mentioned. The difficulty with legislating something that is a structural change is then it is there, and if you want to move and have the flexibility that virtually all of us alluded to, that would only create a different model that would be as inflexible as the concerns we have now with the inflexibility of the model. So I would be much more in favor of providing the NIH, through the Office of the Director, the flexibility to do certain things——

Chairman BARTON. If the Congress provides it, it will be done. If we give the authority to do it, and we let the various stakeholders interact, it might not be done. We may create a process that has no end, where obviously if we do it in law, almost by definition it is going to be imperfect, but at least something will be done.

Mr. FAUCI. But I would submit to you, sir, that the authority would be in law, and then I believe, at least in my rather extensive

experience in dealing with the Congress, is that the Congress looks at us carefully, as they should, because we get what we get from the Congress, and that you have ample opportunity in the future once you give the authority to the NIH to be able to be flexible with those changes that if the kinds of flexibility that are evolving are something that you are concerned about, you can get us in front of you and say, well, let's explain that; what are you talking about.

Chairman BARTON. Of course, the reason you have 27 centers and institutes is over time the Congress has dictated that.

Mr. FAUCI. Right.

Chairman BARTON. We have mandated that this or that be set up, so we are the ones who created the structural problem we are trying to address.

Mr. FAUCI. So help us to be able to have the flexibility of fixing it rather than trying to legislate a fix.

Chairman BARTON. That is why I asked the question.

We have got two more directors, if you would wish to comment.

Mr. VON ESCHENBACH. I come from the perspective that the structure really should be driven by function. I think the authority to define functions and to involve trans-institute and center collaborations is something that I think would be very important for the NIH Director to have. That would allow flexibility without dismantling the structure that is there. You could work with that structure. When it is appropriate and necessary for integration, you would be able to create that. When it was most appropriate for those institutes and centers to stay very mission-focused, that would also then be possible.

I think in that regard it perhaps then approaches it not from making structural changes, but making certain that the authorities allow functional activities to be able to occur in a fluid way.

Ms. VOLKOW. I would agree with my colleagues. I think one of the things that we have seen over the past 10 years in science is that the boundaries, the categories, the labels given to specific fields, are no longer so clearly delineated, and so we see a tremendous overlap across areas in science.

The same thing is happening across our institutes even though we are dealing with different diseases. For example, we are starting to recognize that much of the basic knowledge pertains to multiple disease processes. To me the important aspect is how do you ensure an infrastructure that will allow you to optimize the information and resources required in order that you do not become redundant to the point that you are wasting your resources. How to achieve that, though, is not straightforward.

I think that the element to me is not predefining rigid structures, and again I bring forth the concept that my colleagues have voiced of flexibility that will allow us to drive the organization as the new discoveries and the new emerging trends develop. What I think is important is to recognize the need in a scientific organization like the NIH of having that flexibility. And it will not be automatic, so your help will be required in order to, in certain instances, allow it to proceed more easily.

Chairman BARTON. I have a number of questions, and I will submit them for the record.

Mr. BILIRAKIS. I am gathering before I go to Ms. Capps that you all agree in your responses to Mr. Barton, because you went into these very lengthy responses, you apparently feel that some fixing does need to be done. I don't know when you shake your head yes or no to that effect. Anyway, that is what I get out of that.

Mrs. Capps to inquire.

Mrs. CAPPS. Thank you, Mr. Chairman.

If I am not mistaken, I believe Galileo was either excommunicated or threatened with excommunication for daring to posit a fanatical belief that the Earth revolved around the Sun. My colleague who brought up common sense has had to leave, but I wonder, if common sense had dictated, if we would have ever had a man on the moon or if we would have ever undertaken mapping the DNA. I know that most of us here are very supportive of the work that you do and the way in which you do it.

I will start with you, Dr. Zerhouni, but this really relates to any of the people on the panel to explain to us how the peer review process works and why it is considered the gold standard worldwide for determining scientific quality. Some of us get ahold of the grant applications, and they may sound inappropriate when it is one paragraph. Some of this supports science around esoteric projects, but underlying it is the need to understand the millions of Americans who suffer from HIV/AIDS, sexually transmitted disease, sexual dysfunction, mental health consequences of abuse and various hard topics to get hold of. That is what I would like you to address.

Mr. ZERHOUNI. Sure. I will summarize.

What I can tell you is one of the most common questions I get as I travel around the world, how is our peer review process so effective in identifying areas of science. Over the years, as you know, we have had over 105 Nobel Prizes that have come through the peer review process. The process is as follows: We have two systems that work in succession and sometimes in parallel. There is a center for scientific review which is independent of the institutes, so Dr. Fauci or Dr. von Eschenbach do not directly control the reviews that are done for grants in NCI or NIAID that go through the center for scientific review. So when a scientist proposes an idea, it goes to that center, and that center combines multiple review sections that are categorized according to fields of science.

In 1999 this was reviewed and restructured, because science evolves. So we have review sections which are made up of members which are under the FACA rules, the Federal Advisory Committee Act rules, and the members have to represent a diversity of regions, disciplines, and gender. The members rotate every 4 years.

So those sections are the ones who do what we call the first review, and they score the grants according to scientific merits. Our administrators then compare all the scores across and give a percentile ranking. Those grants then go to the institutes and our Center for Scientific Review will look at a grant and will say this is most appropriate for cancer, or this is most appropriate for NAID or NIDA. It will then go there and undergo the second level of review, which is the advisory council of the institute. The advisory council is, again, a FACA committee made up of usually 18 mem-

bers, 12 scientific members and 6 public members, and they have the final say in what gets funded or doesn't get funded.

They can't not fund things that have received high review. They can also fund things that are at the borderline of grants.

The second—this is about 60 to 70 percent of our grants come through this—the second is what we call special initiatives, where there are special review programs that are organized by the institute. So Dr. Fauci organized last year a competition for having universities create biodefense research centers.

In this context it is such a specialized initiative, that the NIAID puts together an independent peer review panel focused on that area.

For example, Mr. Brown was mentioning muscular dystrophy. If we have a special competition for muscular dystrophy center, that will be reviewed by a special emphasis panel. So 70 percent is independent of the institutes, done independently by scientific review, reviewed again at the advisory council. Thirty percent is done by the institutes, or thereabout, and then reviewed at the advisory council as well.

Mrs. CAPPS. And at some level the public has representation on those committees as well, and all of this—are the names of people on the screening committees, are those made public?

Mr. ZERHOUNI. Right. The names of all the study review panel members are made public. The first degree of review, which is a scientific review, is not open to the public. The second is always open to the public. All advisory councils are open to the public.

Mrs. CAPPS. And then finally——

Mr. ZERHOUNI. I am sorry. But the scientific review portion can be closed to the public; but the members are known, who participates and how.

Mrs. CAPPS. The members are well known?

Mr. ZERHOUNI. They are known.

Mrs. CAPPS. And the professional community respects this. Is this an internationally understood process that is accepted worldwide, or understood at least?

Mr. ZERHOUNI. I can tell you I just—the latest communication I had is the Chinese Government wants to create an NIH in China, and one of the first questions was, tell us how to organize peer review. We get that all the time so it is the gold standard of review worldwide.

Mrs. CAPPS. Thank you. I yield back.

Mr. BILIRAKIS. Mr. Greenwood to inquire.

Mr. GREENWOOD. Thank you, Mr. Chairman. Hello to all.

Dr. Zerhouni, this is a very basic question, and perhaps naive, but something I don't fully understand. I think the number is something like 80 percent of the dollars that flow to NIH then continue out to the universities and health centers and so forth, and something like 20 percent remains inside.

What fundamentally distinguishes the research that is done inside NIH versus outside?

Mr. ZERHOUNI. That is an excellent question. The reason why NIH has created what we call an intramural program was to address historically issues of public health which could not be ad-

dressed. There was no research capacity. There was no talent out there to really address it.

Let me give you a specific example: safety of the blood supply. In the 1960's, you may remember, all the blood collection agencies and so on, and in the 1960's the rate of transmission of a disease through transfusion was 30 percent. We didn't know about hepatitis B and C and all the infections that could be carried through blood transfusions. It was clear at the time that you needed a very dedicated government-driven process to understand all these viruses, and Dr. Harvey Alter has led this program over 30 years.

You can't do this in the system of extramural granting, where every 5 years you have to come in and have your grants reviewed, and if it is a process that takes years, you can't fund it. So typically that is the——

Mr. GREENWOOD. I have three questions I am trying to sneak in here in 5 minutes. Do I not get 8 minutes, Mr. Chairman? Don't I get 8 minutes instead of making an opening statement?

Mr. BILIRAKIS. No. You get 5 minutes.

Mr. GREENWOOD. The investor-initiated applications of research. On the one hand we pretty much have decided as a matter of congressional policy that we try to minimize the micromanagement. We don't want to say, listen, we have had constituents ask us to have more research done on this rather than that, and we have left it to the peer review process.

On the other hand, I am not—when the requests for research are coming from the research community, to some extent that is a function—what they want to study is a function of what they want to study, not necessarily a function of what needs to be studied. So how does your process have an overarching plan and still respond to the not exactly random but somewhat random inputs?

Mr. ZERHOUNI. I will make three short comments. No. 1 is other governments have tried to more micromanage research worldwide, and it hasn't worked. The pharmaceutical industry is a good example of how you do targeted research. They spend twice as much money than NIH and we hear about the productivity of that. So whenever you focus energy at a difference from what the scientists themselves know they can do, you have a loss of efficiency.

How do we know it is really relevant? Well, look at the system. We fund primarily academic health centers. Well, you get promoted because you make a difference in life. You don't get promoted because you are studying some, you know, disease of the right toe. You are trying to solve cancer and you are trying to—that is how the system has a culture that pushes our investigators toward relevant questions.

At the review panel, the public relevance of the program is a component of the evaluation. So it is relevant to public health needs as expressed both by NIH and by the CDC or other components. That is how the integration gets done.

Mr. GREENWOOD. One more question——

Mr. FAUCI. Can I answer? You asked the question, how does research that may be important get done if the investigators don't want to do that? We have been faced with that in the early years of HIV-AIDS when no one was interested in it and in the early years which we are in right now with biodefense. There is a mecha-

nism called a request for application and a request for proposal, which are program-driven, that we are interested in this area. We let the investigators come in with their own creative ideas, but we tell them we have money that we want to invest in this area, and that is how you get people involved in things that they may not otherwise spontaneously get interested in.

Mr. GREENWOOD. Got it.

Last question, what my oversight investigation had on. How do you make sure that there are no conflicts of interest between your reviewers who are deciding which grants get approved if they may have consulting arrangements?

Mr. ZERHOUNI. Right. So all appointments to these review panels are under the Federal Advisory Committee Act rules, so every appointee is a temporary government employee and subject to all of the disclosures of conflict of interest.

So the way we do it, if you are a member of those sections, you have to disclose all of your financial arrangements; not publicly disclose, but disclose internally. If you have a conflict, you are recused from—a good example of a conflict is a case that—a grant gets reviewed from a university at which you yourself are a faculty member. You get excluded from those. So it is the regular processes that we use as mandated by the Federal rules.

Mr. GREENWOOD. Thank you. Thank you, Mr. Chairman.

Mr. BILIRAKIS. Mr. Stupak to inquire.

Mr. STUPAK. Thank you, Mr. Chairman. Thank you to our panel, thank you for coming and testifying today. I think NIH does a remarkable job and look forward to working with you on the many endeavors you undertake. And I think it is just great that our country invests in life-saving and life-better work at NIH.

I am particularly interested, though, in ensuring that the American public has access to safe and effective drugs. Dr. Zerhouni, as you know, back in 2001 Congress passed the Best Pharmaceuticals for Children Act to authorize the 1997 law to grant patent extensions to drug companies in exchange for doing pediatric safety and effectiveness studies.

I opposed this legislation, because I think we have got it backwards. I think it is wrong that we continue to grant these patent extensions once they do a study. I think two things should occur, and I am going to ask your opinion on it. Not only should they do the study; should they not change the labeling on the medicine, on its effectiveness or in effectiveness or safety concerns of children before you grant the extension of a patent?

Mr. ZERHOUNI. Let me make sure I understand the issue. You are suggesting that the drug should be labeled not for pediatric use prior to——

Mr. STUPAK. Granting the extension of the patent.

Mr. ZERHOUNI. I do not know how to answer that question. This is really an FDA-type of authority, but I will really look it up and respond to you on the record.

Mr. STUPAK. Well, we show on average it takes them 14 months after they get the extension to change the labeling. That is 14 more months that we put children at risk—health at risk.

Let me ask you this question, then. The act also gave, and NIH was mandated to research certain on-patent and off-patent drugs.

How many off-patent drugs need to be studied? Do you have any idea?

Mr. ZERHOUNI. The list is made up in coordination—if I——

Mr. STUPAK. With the FDA?

Mr. ZERHOUNI. With the FDA and NIH. The list is primarily the responsibility of NIH.

Mr. STUPAK. Okay.

Mr. ZERHOUNI. And then this list is used in our BPC program——

Mr. STUPAK. Do you have any idea how many are on this list?

Mr. ZERHOUNI. No. I don't have the exact number.

Mr. STUPAK. How about the on-patent drug——

Mr. ZERHOUNI. I will provide you that number, sir.

Mr. STUPAK. Is it safe to say, given the resources available to you today, that a lot of these drugs that need to be studied are not being studied?

Mr. ZERHOUNI. That is a fair statement.

Mr. STUPAK. Didn't NIH just complete a report on the gap on the on-patent and off-patent studies and what needs to be done? Did you not just do a report?

Mr. ZERHOUNI. We actually did review the BPCA implementations. So there is a report that I think we sent to Congress, if I am not mistaken, but I will——

Mr. STUPAK. We haven't seen that yet. Could you provide that to this committee, because I would like to see those numbers and what drugs are and are not——

Mr. ZERHOUNI. Definitely.

Mr. STUPAK. The Best Pharmaceuticals for Children Act also included a provision to create an independent foundation. It was called a foundation for pediatric research, to collect funds and award grants for research on on-patent drugs when the drug companies do not want to do the studies themselves. And, again, I think those results have been submitted to you and to the FDA commissioner.

Could you also provide us a copy of that?

Mr. ZERHOUNI. I will.

Mr. STUPAK. In choosing the studies on on-patent drugs, the ones that are targeted to be studied, what is your involvement in it, or NIH's involvement in it?

Mr. ZERHOUNI. Primarily looking at the importance of the drug and how utilized is it in the population under—you know, the pediatric population. You will see that, for example, one area that NIMH, the National Institute on Mental Health, is very interested in is the use of antidepressants, and that has become a major issue; so institutes have a way to weigh in and give their advice in terms of what they think should be tested.

FDA, however, has the primary role, because if they receive information about it, adverse events, and they know what post-market surveillance data is available for——

Mr. STUPAK. On the antidepressant, actually, with children lately, we have been seeing a lot of reports on that. So what involvement would you have on that? I mean, there has been a lot of controversy with the British studies and one gentleman at FDA not being allowed to testify publicly. What involvement would you as

NIH say to the FDA to try to get these studies out and get them up there?

Mr. ZERHOUNI. Directly, I don't have a lot of personal involvement in it. But NICHD, which is our lead institute for children's research, is the lead, and that is the institute that really interacts with the FDA.

Mr. STUPAK. And who heads that institute?

Mr. ZERHOUNI. Dr. Duane Alexander.

Mr. BILIRAKIS. The gentleman's time has expired.

Bart, do you have something you can go through real quickly?

Mr. STUPAK. They are pretty long ones. I will submit the rest of them in writing. And if you would just submit those studies to us, I would appreciate it, especially on the on-patent and off-patent drugs.

Mr. ZERHOUNI. I will certainly do that.

Mr. BILIRAKIS. Mr. Pitts to inquire.

Mr. PITTS. Thank you, Mr. Chairman.

First I think I ought to try to make a clarification. Some of my colleagues on the other side seem to question the intent of some members who question NIH on the merits of particular studies, and the position of the other side seems to be that sexually transmitted diseases affect a lot of people, and that is why it is an important study. And I want to be clear; no one ever questioned whether STDs are important to study. I think we all agree with that.

What some question was how paying people to watch pornography is related to STDs and how is it related to HIV-AIDS, and how, as one NIH spokesman claimed, it has anything to do with abstinence education.

I think we all agree that we need to find a cure for HIV-AIDS, but there is some question about what is the best way to do it; is there a better way than paying people to watch pornography? That said, let me move on to my questions.

Over the years there have been questions raised about some of the grants that NIH has awarded and whether or not these grants were appropriate uses of taxpayer dollars. And while some grants arguably have scientific value that may not be apparent to a layperson, there are also some grants that are arguably of questionable priority when reviewing the entire NIH research portfolio.

And, Dr. Zerhouni, I am pleased to hear that you are working toward more transparency issues, as you stated. And I want to be clear; I prefer not to go grant by grant and bring congressional grants up at every hearing, but I can't help but mention one. I saw a study of dorm-room wall decorations, Web pages of college students. It was funded 1 year.

How can we convey to constituents, taxpayers, who have family members with Parkinson's or leukemia or diabetes, who are concerned about ensuring the safety of the blood supply, that studying dorm-room wall decorations was more worthy than finding a cure for their diseases? When a multiyear grant is awarded by an institute, what if any authority do you have as NIH director to make a change if it is determined at a later date that this project is of less significance, given current public health needs?

Have any of you ever stopped funding of an awarded grant based on an unexpected budgetary need? And, Dr. Zerhouni, you can start.

Mr. ZERHOUNI. Sure. Obviously, this is an issue. As I said, we need to work on and make sure that we review these grants. The dorm-room grant study was funded over 3 years ago, is no longer active. I looked into that because of the questions that were raised, and what I am told is that the reason the study was performed is that you can tell the likelihood of mental health disorders according to the decoration that student in a college will put up in their room and what kind of analysis—personality analysis this will be.

So the way I understand why the research was thought useful at the time by the committee that reviewed it was that it could provide you with a diagnostic test of children, students—college students that may be getting in trouble from the mental standpoint, and their personality disorders and so on. This is what I am told was the reason for that.

But the question you are asking is a much more profound question, and that is relative allocation of resources. And that is something, as I mentioned, to do that better, I think you will need to have, A, a better understanding of the portfolio and the relative importance of the portfolio, and this is why I suggested that we need to have a portfolio review mechanism, both within institutes and across institutes, because each program obviously evolves over time, and has the budget that they need to spend according to both the primary and secondary review.

So I want to make sure that we have processes in place that will give you the assurance that it has been reviewed, that its scientific merit is established, that it is explained in clear terms so that there is no—like, if you read this title, obviously it makes no sense, but if you look into it, you realize that psychological tests that look at drawings, for example, that children use on their wall tell us something about the mental state of an individual. And this is something you can argue in terms of is it right to spend that dollar on that thing instead of spending it on something else. This is why I suggested that we need to have better mechanism.

The authority that the director has to stop a grant is, I would say, very limited; because if it has passed review, has been approved by the advisory council of an institute, unless two things happen, one, that the progress reports indicate that the research is not making any headway or that, two, there is an inappropriate use of Federal funds, we will terminate the studies. That is pretty much the authority of the director of the NIH, but I will let my colleagues comment as well, because they have greater authorities within their own portfolio.

Mr. BILIRAKIS. Very briefly, if you would like to comment.

Mr. VON ESCHENBACH. Just to give you a specific example of the importance of managing the portfolio and making shifts to strategic priorities, one of the things we did this very year was make a decision with regard to a study that had been going on with regard to looking at mammography and its utilization and standards for interpretation. That study was coming up for reissuance and rereview, but the analysis indicated that we had received significant input and information about the outcomes of that question that

was first raised, and essentially that grant had reached its fulfillment. And rather than reissue it and continue it on, we stopped that and redeployed those dollars to other initiatives where we can look at even more effective ways of detecting breast cancer earlier. So we do make those kinds of strategic shifts in decisions.

Mr. FAUCI. Another example would be human subjects issues. We have stopped grants that if you look at the design of a clinical trial, for example, in a developing nation, that although the science would be compelling to get the answer to the question, it could not be done under the proper ethical circumstances, so we would stop it even though it got a good scientific score. I have done that.

Mr. BILIRAKIS. The gentleman's time has expired. . And then I appreciate the patience the gentleman has shown throughout the entire hearing.

You know, Mr. Waxman made a—focused on keeping politics out of science and research, and I will tell you, and I have said this before—I know Dr. Zerhouni at least has heard me say it a number of times—I think probably maybe the toughest part of my job—and I will bet I am speaking for Mr. Brown and virtually every member of this committee—is when someone comes wheeling into our office as an ALS patient and tells us that there isn't enough research on ALS and then knows about some of these other areas that Mr. Pitts and others have mentioned. I lost my youngest brother to Parkinson's in his mid-fifties, and you know where—Mohammed Ali has come in and testified.

I think I am losing something here. I think I need some NIH right about now.

I guess what I am saying is it is a tough thing for us to tell them, thank you; to tell them that we believe very strongly that we should not micromanage; we believe very strongly that we are not in a position to determine where the funding should go in terms of what specific disease, things of that nature. So we are staying out of it. But I would also strongly suggest there is probably a hell of a lot more politics being played within NIH and among the institutes than ever comes out of the Congress in this regard.

Whether you agree with me or not, I don't know, but I think if you kind of search your experiences, you would find that that is the case.

So we intend to continue to do this tough thing of telling these many patients, these people who come in here and testify, that we don't think it is our role to determine what funding should go—research funding should go to what disease, but we also like to think that you are helping us in that regard, too, through some of the grants.

I mean, the word "common sense" was used, and issue was taken with that by another member and that sort of thing. I don't know what is right and what is wrong. All I know is, put yourselves a little bit in our shoes when we have to tell these people and they know what some of this funding award is going to.

Your explanation in terms of the painting of the dorm walls and whatnot, well, I can see where there is something behind that. But when you have that patient out there who is dying, I think they would rather see that money, rather than go to studying the painting of those walls, go into something involving ALS or whatever.

So that is the position that we are always in, and I appreciate your coming here today. I think you have worked awfully hard to answer our questions and our inquiries. I am not sure Mr. Brown is satisfied as far as Dechenne muscular dystrophy is concerned. For instance, I am not sure that any of us are completely satisfied, but transparency I think is the answer, and we depend upon you for that. And you are just the magicians in our society, your research and all your work and whatnot. And all of us are strongly supportive of that, contrary to some of the statements made in this hearing up here.

We have a number of questions, as usual, to submit to you. We would appreciate it—as you heard Mr. Barton say, we haven't reauthorized NIH for quite a few years. It hasn't affected your work, because you have gotten the money, but it looks like we may be advancing toward that. I mean, discussions have taken place between the minority and the majority on maybe some issues and things of that nature. So I guess what I am saying is, no, we shouldn't take the flexibility away from you, in my opinion, but we also need recommendations from you on what we should be doing that you would need to see legislative changes in order to take care of some of the problems that you all know of much better than we do.

Mr. Brown, do you have any closing statements? Please feel free.

Mr. BROWN. Only to add that—and thank you, Mr. Chairman—I appreciate this hearing, and I always appreciate so much what NIH has done. I hope we can get our government to pay the same attention to the CDC that we do the NIH, but I also hope that we don't make decisions in the next few years that cripple this Nation's ability to do research, not just the research that you do but also research with—we are doing better with math and engineering and where we go in that direction. And I am afraid, just as we look at budget questions that come about because of policies that people in this Congress make, that we think more of the future than we seem to be thinking right now. And I applaud all four of you and applaud all your colleagues out of each institute, and especially the employees of NIH and the contractors at places like Cleveland—in Cleveland with Case Western Reserve University and other great institutions around the country that benefit from the decisions you make, and only the public benefits from the decisions both you and they make. So I thank you for that.

Mr. BILIRAKIS. Thank you very much. Thank you, Doctors. The hearing is adjourned.

[Whereupon, at 4:30 p.m., the subcommittee was adjourned.]

[Additional material submitted for the record follows:]

Enhancing Public Input and Transparency in the National Institutes of Health Research Priority-Setting Process

A Report by
the NIH Director's
Council of Public Representatives (COPR)

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COPR would like to acknowledge the efforts of many individuals in the Institute and Center Offices of Public Liaison who participated in teleconferences and meetings and by e-mail to support this work group's efforts. This report could not have been completed without the hard work and background information provided by Institute, Center, and Office of the Director staff.

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Executive Summary

Engaging the public is a major priority, it is a national priority, it is not an option.
—Elias Zerhouni, M.D.

The National Institutes of Health (NIH) Director's Council of Public Representatives (COPR) was established by the NIH Director in 1998 in response to a need identified in the Institute of Medicine (IoM) report, *Scientific Opportunities and Public Needs: Improving Priority Setting and Public Input at the National Institutes of Health.* The COPR was established in recognition of the need for a forum through which the NIH leadership could interact with representatives of the public. To review the state of public input and participation at the NIH, COPR designated a Public Input and Participation Work Group (PIPWG) to gather information. In April 2003, PIPWG identified the two areas that would be the primary focus of this report: public input and transparency. PIPWG also found it necessary to define the term public. At its most encompassing, the public is everyone outside the NIH. COPR believes that the NIH, in its worthy goal of improving quality of life for all Americans, should facilitate the flow of information to and from *all* members of the public who are interested in a particular subject area. The NIH should also understand that the composition of the public varies, and strive to use the broadest practical definition.

Public Input

Because its $27 billion budget is derived from taxes, the NIH has a duty to educate members of the public about its mission and operations and solicit their input and participation in the research priority-setting agenda. The COPR believes members of the public should be recognized as equal participants in open discussions and should be one essential component in the research priority-setting process. In their examination of the state of public input and participation in the NIH research priority-setting process, COPR identified seven principles as important in reaching the public and facilitating its input.

1. Go Beyond the NIH Campus

The town hall meeting is a proactive outreach method that takes NIH officials off campus to cities and towns nationwide and gives average citizens access to top scientists and decision makers. Several Institutes and Centers use the town meeting strategy to disseminate their message, solicit input, and enhance access to NIH decision makers.

Recommendation 1: Go beyond the NIH campus to engage the American public where they live.

2. Partner with Communities

Forming continuing partnerships with local communities, grassroots organizations, and leaders creates an important conduit for ideas and input that builds lasting relationships. It is no small task to effectively engage local communities, their leaders, and grassroots groups in providing input on the complex issues involved in medical research. Institutes and Centers should seriously consider using best practices for actively seeking input from local community leaders and grassroots organizations, where appropriate.

Recommendation 2: Partner with local communities, grassroots organizations, and community leaders.

3. Use Proactive Outreach, Including High- and Low-Technology Approaches

Proactive outreach involves actively soliciting public input, such as sending e-mails and other information out for comment and distribution. The NIH should consider this approach to enhancing the transparency of its operations and increase public participation. Two NIH entities use media technologies to increase awareness and solicit feedback, and the NIH Director's Office of Communications and Public Liaison has a multimedia kit called the *NIH Talking Points Toolkit*. It is also important to continue to use and not abandon low-tech approaches for communication and comment, like printed material and annual reports.

Recommendation 3: Use proactive outreach, and provide access for non-Web users.

4. Increase Cross-Institute Communication

The Institutes and Centers use Working Groups and Progress Review Groups to focus resources, technology, and scientific opportunity on a specific disease or disorder. The National Heart Lung and Blood Institute, the National Institute of Neurological Disorders and Stroke (NINDS), and the National Cancer Institute (NCI) have all participated in such efforts. NIH staff members have also developed novel resources and methods for public outreach.

Recommendation 4: Develop more partnerships among Institutes and Centers and foster cross-Institute communication on crosscutting diseases or issues. COPR strongly encourages all Institutes and Centers to work together to share information throughout the NIH.

5. Promote Bidirectional Communication

Setting NIH research priorities should be a collaborative process in which the public is involved from the outset. For example, the National Institute of Arthritis and Musculoskeletal and Skin Diseases Community Health Care Center provides health care services related to arthritis, lupus, and other rheumatic diseases. The National Institute of Mental Health Regional Outreach Dialogue meetings are town hall meetings held in locations across the country. The National Institute of Diabetes and Digestive and Kidney Diseases is developing an education program to reduce morbidity and mortality caused by kidney disease.

Recommendation 5: Foster two-way communication and dialogue on an individual level and with communities where research is performed.

6. Make Sure Public Input Reaches Decision Makers

It is important that senior decision makers actively demonstrate a commitment to public input. One way to make this happen is to hold listening circles where directors and senior decision makers are present and can hear public input. Listening circles are forums in which people come together to share information and knowledge about a topic. As one example, National Library of Medicine (NLM) listening circles seek to share information about NLM.

Recommendation 6: Ensure that senior decision makers receive and fully consider public input.

7. Fully Utilize Advisory Councils and Their Public Members

Each Institute and Center charter mandates that persons who represent the general public fill a certain number of advisory council seats. Beyond the selection of advisory council public

members is the issue of identifying those members as such. The National Institute on Deafness and other Communication Disorders, NCCAM, and NINDS routinely consult their advisory councils in the priority-setting and planning processes. The COPR urges Institutes and Centers to enhance the role of public members on Institute and Center advisory councils and consider creating new mechanisms to empower them.

Recommendation 7: Ensure that advisory council public members represent a broad range of public constituencies and that the councils are fully used as an important avenue for public input.

Transparency

In considering public input into the research priority-setting process at NIH, one issue warrants special focus—transparency. To study the transparency of the NIH research priority-setting process, COPR examined the activities and practices of various Institutes and Centers. In general, the results of these interviews were consistent with and significantly confirmed major conclusions and observations reached as a result of COPR's other investigations. In considering the goal of enhancing transparency, three types of activities were identified as fundamentally important: education, access, and active listening. It is not enough that transparency exists in the system in the form of opportunities for education and points of access.

8. Educate the Public about the NIH

The National Institute of Child Health and Human Development (NICHD) NIH 101 program helps educate the public about the NIH. NIH 101 includes a brief history of NIH and NICHD, information about how budgets and research priorities are set, a breakdown of the grant review process, information about how NICHD communicates research findings, and instructions for finding other NIH information. NICHD should be applauded for its innovation and initiative in engaging groups and responding to requests for information.

Recommendation 8: Actively develop tools and materials that help educate the public about the research priority-setting process and opportunities for public input.

9. Enhance Access

One transparency-enhancing activity that improves public access is the NCCAM Stakeholder Forums. During the forums, participants testified about their experiences and opinions related to developing the NCCAM Strategic Plan. Another example of improved access is the www.getinvolved.nih.gov Web site. NIH Institutes and Centers contribute information about public outreach events, public resources, and special public announcements. Such continual improvement of access for members of the public to learn about input opportunities and provide input should be maintained and supported.

Recommendation 9: Continue to search for mechanisms that encourage public input into the research priority-setting process and that are easily accessible and provide information-sharing opportunities.

10. Practice Active Listening

A transparency-enhancing activity related to active listening is the 2003 Survey of Cancer Advocacy Organizations conducted on behalf of the NCI and the NCI Director's Consumer Liaison Group (DCLG). The survey solicited information about each organization and its familiarity with NCI, thoughts about future DCLG direction, and preferred communication

methods. NCI transparency was not the survey's main purpose, but some information was directly relevant. The survey was laudable in its use of an approach that actively solicited information from this sector of the public.

Recommendation 10: Actively solicit information from constituents and the general public about the public's experiences and perceptions of transparency at the NIH.

Support and Funding

11. Provide Adequate Resources

Implementing some of the above recommendations will require a significant investment of funding, staffing, and support.

Recommendation 11. Provide adequate resources in terms of funding, support, and staffing to allow for the successful accomplishment of these recommendations.

Conclusions

The guarantee of public input and participation in the NIH research priority-setting process and the transparency of that process are essential to promoting public trust in the research enterprise. The COPR recognizes that significant opportunities exist for public input and transparency that are not identified in this report. Support for and belief in the importance of public input into the NIH research priority-setting process must be embraced from the top down. It is clear that many Institutes and Centers at the NIH take the issue of public input and transparency seriously, but more could be done.

Introduction

Never doubt that a small group of thoughtful committed citizens can change the world. Indeed, it is the only thing that ever has.

—Margaret Meade

The National Institutes of Health (NIH) Director's Council of Public Representatives (COPR) was established by the NIH Director in 1998 in response to a need identified in the Institute of Medicine (IoM) report, *Scientific Opportunities and Public Needs: Improving Priority Setting and Public Input at the National Institutes of Health.*[1]

The COPR was established in recognition of the need for a forum through which the NIH leadership could interact with representatives of the public. COPR is the primary mechanism by which public representatives can provide their perspective on matters of public interest and work with NIH leadership to increase public awareness of NIH and how it functions. The COPR is the primary mechanism for providing public input to NIH decision makers at the highest level. In recent efforts, COPR identified several areas in which the Council might be uniquely effective in helping the NIH Director understand the public perspective.

The IoM report examined four issues related to setting research priorities at NIH: allocation criteria, the decision-making process, the impact of congressional directives, and mechanisms for public input into the research priority-setting process. In the four years since the release of the IoM report, NIH has enhanced many activities related to public input and participation. In this 2004 report, COPR examines the results of those changes and makes recommendations for improvement.

To review the state of public input and participation at the NIH, COPR designated a public input and participation work group (PIPWG) to gather information. The work group began by educating itself about the flow of information between NIH and the publics it serves.

Between October 2002 and April 2003, through the work of PIPWG and with much collaboration with the Institute and Center Officers of Public Liaison (OPLs) and Communication Directors, COPR determined that many opportunities exist at the Institutes and Centers for transmitting information to the public. COPR also determined that there is substantial disparity between public participation at NIH in general, and public participation in the research priority-setting process identified in the IoM report. Further, the public tends to perceive the NIH research decision-making process as "secretive," difficult to understand, and ambiguous, resulting in a serious lack of transparency at the NIH from the public's perspective.

In April 2003, PIPWG identified the two areas that would be the primary focus of this report:

- ***Public input.*** Identify best practices for obtaining and using public input and participation in the research process.
- ***Transparency.*** Consider practices that enhance public examination and awareness of the *process* of making research choices and that enhance the access of senior NIH decision makers to public input into this process.

During this time, PIPWG also frequently found it necessary to define the term public. In reality, there are many types of publics. At its most encompassing, the public is everyone outside the NIH. More specific publics may include groupings of the following:

- Patients
- Patient advocates
- Constituency groups and organizations
- Nonprofits
- Health care providers
- Health care practitioners
- Investigators
- Research organizations
- Educators
- Media
- Congress

COPR believes that NIH, in its worthy goal of improving quality of life for all Americans, should facilitate the flow of information to and from *all* members of the public who are interested in a particular subject area. In facilitating this information flow, NIH should take special care to accommodate those who do not have ready access to computers.

NIH should also understand that the composition of the public varies, and strive to use the broadest practical definition. COPR urges that NIH carefully and regularly monitor which public is being served with regard to a particular subject and determine whether patients, patient advocates, constituency groups, and related organizations have appropriate roles. COPR believes these publics can and should play a broader role and that NIH should tailor its activities appropriately.

Public Input

Because its $27 billion budget is derived from taxes, the NIH has a duty to educate members of the public about its mission and operations and solicit their input and participation in the research priority-setting agenda. Methods for accomplishing this goal differ based on the needs of diverse constituency groups that work with the 27 Institutes and Centers. Some Institutes and Centers are more proactive and targeted than others, depending on several factors, including the nature of their mission, the constituencies they serve, and the amount of their appropriation.

A proactive approach gives the public a greater awareness of NIH's desire for and openness to public input, and ultimately works to enhance transparency by promoting greater public awareness of and involvement in the research enterprise.

The vision of the IoM report[1 (p. 61)] was that the COPR recognize and identify "*channels through which the public can provide broad input into the NIH priority-setting processor or through which NIH can respond clearly and authoritatively to the public on issues of mutual concern.*"

The COPR does not envision nor does it intend that members of the public are the sole determinants of the research priorities that are chosen at each Institute and Center. Rather, the COPR believes members of the public should be recognized as equal participants in open discussions, and one essential component in the research priority-setting process.

Improving Input at NIH

In their examination of the state of public input and participation in the NIH research priority-setting process, COPR identified the following seven principles as important in reaching the public and facilitating its input. Cited are some examples of best practices that represent these principles as carried out by selected individual Institutes and Centers. It should be noted, however, that this is in no way an exhaustive attempt to identify all effective practices conducted at all Institutes and Centers.

1. Go Beyond the NIH Campus

The town hall meeting is a proactive outreach method that takes NIH officials off campus to cities and towns nationwide and gives average citizens access to top scientists and decision makers. The public hears firsthand about NIH initiatives, and decision makers receive direct feedback about NIH work.

Several Institutes and Centers use the town meeting strategy to disseminate their message, solicit input, and enhance access to NIH decision makers. This strategy has been so successful for the National Institute of Environmental Health Sciences (NIEHS) that it recently published its experience with town meetings in the journal *Environmental Health Perspectives.*[2] The NIEHS found town meetings to be "a successful model for bringing academic researchers together with community residents, state and local departments of health, and community-based organizations to foster greater awareness of community needs, public health needs, and environmental health science research." Since 1998, the NIEHS has sponsored 16 town meetings nationwide and strongly supports this avenue for public input.

Another Center that has used the town meeting approach is the National Center for Complementary and Alternative Medicine (NCCAM). NCCAM has held three town meetings nationwide in collaboration with existing NCCAM research centers. The purpose of these town meetings was to give the public an opportunity to learn about the latest research being conducted and ask questions, and for the Institute to answer questions and obtain input from the public. The meetings were attended by the Institute Director and included an address by him. There were 700 to 800 attendees at the most recent meeting.

The benefits of going beyond the NIH campus include:

- Receiving ideas from a new perspective.
- Creating new methods of working with the community.
- Strengthening public good will and support.
- Enhancing public access to NIH decision makers.

> **Recommendation 1: Go beyond the NIH campus to engage the American public where they live.**
>
> **NIH Institutes and Centers should take their message to and solicit ideas from the American public through the use of regional forums and town meetings. The NIH Office of the Director and Institutes and Centers should collaborate to reduce costs.**

2. Partner with Communities

Forming continuing partnerships with local communities, grassroots organizations, and leaders creates an important conduit for ideas and input that builds lasting relationships.

Much is said in the NIH Roadmap for Medical Research about the need for collaboration between important entities to advance the research enterprise. An element of such collaboration that requires involvement but is sometimes overlooked is partnering with local communities and grassroots organizations. A grassroots organization is a group that is in touch with members of the local community. Especially important are groups from rural or non-urban areas that often represent particular interests of local community members.

It is no small task to effectively engage local communities, their leaders, and grassroots groups to provide input on complex issues like medical research. A commitment is needed and resources must be expended to ensure that grassroots organizations, their leaders, and their communities are consulted. They must be assured that their input will be thoroughly considered and incorporated.

Several Institutes and Centers underscore the value of such involvement in their research-setting process. One example involves the work of the National Eye Institute and the National Library of Medicine. Both held listening circles in areas where the predominant population was Native American and Native Hawaiian. The goal of these meetings was to seek input from constituencies that are traditionally underserved but represent an important viewpoint.

Another NIH effort to partner with population groups at a local level involves the National Cancer Institute (NCI). The NCI, through grant funding, sponsored the development of a video program to help physicians learn culturally sensitive communication techniques for

talking about cancer and the importance of clinical trials with low-income African Americans. The project centered on direct focus group input from public housing residents in Cleveland, Ohio.

The benefits of partnering with local communities and grassroots organizations include:

- Receiving immediate feedback on important issues.
- Creating a sense of connection with policy and decision makers.
- Forming an open conduit for continuing dialogues.
- Accessing viewpoints from those outside the mainstream.

Institutes and Centers should seriously consider using the best practice of actively seeking input from local community leaders and grassroots organizations, where appropriate. Significant and important ideas exist beyond the NIH campus that decision makers should hear. Cost considerations may present a challenge but, by working together wherever possible, Institutes and Centers may be able to manage the associated costs.

> **Recommendation 2: Partner with local communities, grassroots organizations, and community leaders.**
>
> **NIH should consider forming or enhancing partnerships with local groups and leaders to help foster an atmosphere in which ideas and input can be shared.**

3. Use Proactive Outreach, Including High- and Low-Technology Approaches

Proactive outreach involves actively soliciting public input, such as sending out e-mails and other information out for comment and distribution. Proactive outreach increases opportunities to build closer working relationships by taking NIH access to the public, not waiting for the public to come to the NIH. New technologies create new opportunities for soliciting public input; COPR encourages aggressive use of these pathways.

In 1979, telephone company ads encouraged the use of long-distance telephone service to "reach out and touch someone." NIH should consider the same approach in its effort to enhance the transparency of its operations and increase public participation. By using accepted, proactive public outreach tactics such as push e-mails, direct mail, response cards, and videotapes, the NIH can solicit input related to the research priority-setting process.

A growing number of Americans rely on the Internet to acquire information, do business, or stay in touch. According to a February 2004 survey by the nonprofit Pew Internet and American Life Project, 63% of American adults have access to the Internet. A July 2003 survey by the same group indicated that 80% of American Internet users have searched online for information on at least one of 16 major health topics. Based on these statistics, a successful organization would try to engage this growing, technologically astute population segment to solicit feedback and ideas or build organizational awareness. The NIH should use these tools to strive for continuity with the public, and develop standard practices to make public interaction user friendly and build closer working relationships with constituency groups and the public.

Two NIH entities already take advantage of media technologies to increase awareness and solicit feedback. The NIH Director's Office of Public Liaison is developing a constituency

outreach database to build organizational awareness on activities and topics related to central NIH issues. This database will provide information to targeted and broad-based constituencies and NIH stakeholders through push e-mail. The content will include information on the NIH Roadmap for Medical Research, the evolving NIH Public Trust Initiative, and special staffing alerts from the NIH Director.

In addition, the NIH Director's Office of Public Liaison has a multimedia kit called the *NIH Talking Points Toolkit*. The kit was originally designed as a guide for COPR members who spoke to groups about the NIH. COPR believes its use might be broadened and expanded as a resource for the public that could be adapted by each Institute. The boxed kit's centerpiece is a professionally produced videotape that chronicles the NIH history and mission and helps convey the importance of NIH's work to outside groups. The video is accompanied by paper copies and disk copies of NIH 101 and COPR 101 PowerPoint presentations, a disk copy of the NIH Almanac (a multimedia resource), and resource lists of NIH Web links that include information such as toll-free numbers for questions about specific diseases; Web site locations for learning more about NIH-funded institutions, research centers, hospitals, and universities by state; and highlights of recent NIH research advances.

The National Institute on Alcohol Abuse and Alcoholism (NIAAA) is using the Internet to solicit feedback on its annual report. This effort uses push e-mails, which are distributed to people on a specific mailing list to invite them to read the annual report online and comment on it. The e-mail describes the project's background, states the intent to solicit an opinion, and includes the annual report's Web address. This has been a successful tactic for NIAAA.

Additionally, it is important to continue to use and not abandon low-tech approaches for communication and comment, like printed material and annual reports. While the Internet provides an instant means for bidirectional communication with a large portion of the public, traditional modes of communication remain an effective method for engaging important segments of the public that may be less involved with newer technologies. Maintaining a low-tech approach while exploring newer communications strategies and technologies will ensure broad public involvement.

The benefits of using proactive outreach include:

- Building trust through transparent efforts to seek and consider the public perspective.
- Gaining immediate feedback from targeted groups.
- Increasing public input with maximum cost efficiency.
- Enhancing and developing new relationships with constituency groups and the public.

The best practice of increasing the use of innovative public outreach techniques and using them proactively will strengthen NIH transparency and benefit the institution and the public.

Recommendation 3: Use proactive outreach, and provide access for non-Web users.

New technologies provide more opportunities for soliciting public input. NIH is encouraged to continue and enhance aggressive proactive outreach to the public, such as sending out e-mails and other solicitations for comment. At the same time, NIH should create alternative outreach methods to Web-based systems, such as printing and mailing annual reports to constituency groups for comment.

4. Increase Cross-Institute Communication

The Institutes and Centers use Working Groups and Progress Review Groups to focus resources, technology, and scientific opportunity on a specific disease or disorder. The Neuro-Oncology Program by the NCI and the National Institute on Neurological Disorders and Stroke (NINDS) is an example of cross-Institute collaboration at NIH.

Research into many diseases is carried out simultaneously at several Institutes and Centers. Collaboration is generally desirable but does not always occur in individual laboratories. Working Groups and Progress Review Groups bring together scientists and clinicians from many disciplines and intra- and extramural institutions. NIH emphasizes investigator-initiated proposals and research, but collaborative work on specific diseases has had significant results. Including public members in these bodies offers a critical perspective and demonstrates the strong interest and shared commitment that patients, their families, and other members of the public have in addressing issues and solving problems.

In 2002, the National Heart Lung and Blood Institute organized a workshop on sarcoidosis, a disease that causes inflammation of the body's tissues. Investigators and clinicians from other Institutes and Centers and from universities and research centers nationwide were invited. A sarcoidosis Patient Interest Organization (PIO) was also invited to participate. These representatives attended an evening presentation before the workshop to learn about the most recent treatment advances. During the workshop the next day, the representatives shared patient perspectives and added to the information available to scientific members.

From 2000 through 2003, NINDS supported a Public-Private Working Group devoted to Parkinson's disease. This group meets annually in person and telephones biweekly to discuss research advances and priority-setting processes. This group includes NIH scientific staff, industry representatives, patients, and caregivers.

In 2000, the NCI and NINDS jointly held a broadly focused Brain Tumor Progress Review Group. Attendees included NCI and NINDS senior staff and researchers, and clinicians from both Institutes and from U.S. and Canadian universities and medical centers. Patients, patient advocates, and industry representatives were also invited. A priority of the Progress Review Group was to improve communication among the represented communities and examine the state of the research.

NIH staff members have developed novel resources and methods for outreach to the public. In 2002, the Offices of Public Liaison formed a Regional Outreach Work Group to identify ways to enable NIH outreach staff to share information on regional outreach efforts, best practices, and lessons learned.

The benefits of using Working Groups and Progress Review Groups include:

- Bringing the public perspective directly to scientists and decision makers.
- Enhancing trust among researchers, patients, families, and groups.
- Facilitating information sharing across NIH.
- Promoting the replication of best practices and successful activities across NIH.

Recommendation 4: Develop more partnerships among Institutes and Centers and foster cross-Institute communication on crosscutting diseases or issues. COPR strongly encourages all Institutes and Centers to work to share information throughout NIH.

NIH should consider developing a mechanism similar to Working Groups and Progress Review Groups that promote sharing resources, technology, and scientific opportunity on a specific disease or disorder. Tools and resources for sharing information such as those developed by the Regional Outreach Work Group are only as useful as the information that is provided by the individual Institutes and Centers. To foster cross-Institute communication, each Institute and Center must commit to active participation in all information-sharing efforts.

5. Promote Bidirectional Communication

Setting research priorities at NIH should be a collaborative process in which the public is involved from the outset. For research priority setting to be truly collaborative, Institutes and Centers must create and maximize opportunities for two-way communication and dialogue with individuals and communities. Three examples of this are the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) Community Health Care Center, the NIMH Regional Outreach Dialogue meetings, and the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) National Kidney Disease Education Program.

The NIAMS Community Health Care Center in Washington, DC, is a medical and health information center that provides health care services related to arthritis, lupus, and other rheumatic diseases. Patients who suspect they have such a disease or who have been diagnosed can receive medical attention by self referral or referral by a health care provider.

Depending on their medical condition, patients are eligible for primary treatment, more advanced treatment and testing, or clinical trials and experimental treatments. In addition to treating an essentially minority population (African-American and Hispanic), the Center raises awareness of health disparities in rheumatic diseases, increases minority participation in research studies, and boosts the number of underrepresented biomedical researchers in this field. Moreover, patients are empowered to become participants in their own research protocols.

The NIMH Regional Outreach Dialogue meetings are a series of town hall meetings held in various locations across the country. They have been held in Anchorage, San Antonio, Chicago, Pittsburgh, and Albuquerque, and have addressed topics relating to mental health issues important to traditionally underserved populations. A primary purpose of the meetings is to promote two-way communication: not just to disseminate information about NIMH research, but to hear public input.

Although the dialogue meetings are one-time events, what is notable is the extensive amount of preparation that goes into each one. Prior to planning a meeting or setting an agenda, several field visits to the area are conducted. During these visits, NIMH staff members engage in a dialogue with local people and representatives of communities of interest. This give-and-take of information is used to discover local concerns and set an agenda, and to identify appropriate individuals and groups to invite to participate. Approximately 300 people participated in the last meeting in Albuquerque. NIMH is represented by the Director and Deputy Director and staff and top researchers in areas of interest for that topic or population.

The NIDDK is developing an education program, the national Kidney Disease Education Program, to reduce morbidity and mortality caused by kidney disease and its complications. Prior to launching a nationwide program, a pilot program is currently being implemented. The pilot program is designed to reach out to African Americans, a group at very high risk for

diabetes, hypertension, and kidney disease. In the process of developing the pilot program, a series of focus groups was convened to identify channels, intervention strategies, and messages that would be most effective in increasing awareness of kidney disease. Additionally, this information was used to develop culturally appropriate and meaningful materials.

The benefits of two-way communication and dialogue include:

- Developing ongoing relationships between the NIH and local communities that foster trust and increase engagement in the clinical research enterprise.
- Developing a truly community-based research agenda shaped by meaningful input and collaboration.
- Increasing the participant investment in the process and outcome.

Recommendation 5: Foster two-way communication and dialogue on an individual level and with communities where research is performed.

COPR encourages NIH to use a collaborative approach with the public whenever possible, in particular with research participants. This involves an ongoing process of two-way communication and dialogue as activities and research are undertaken and research priorities are set.

6. Make Sure Public Input Reaches Decision Makers

It is important that senior decision makers actively demonstrate a commitment to public input. This would ensure that future decisions concerning NIH research and priority setting are transparent, and that public input and participation are solicited.

One way to make this happen would be to hold listening circles where directors and senior decision makers are present and can hear public input. The National Library of Medicine (NLM) holds listening circles with the Native American population and has arranged for these listening groups to be held in the Dakotas and other areas, during which senior NIH officials met with the leadership of the tribal or native nations.

Listening circles are forums in which people come together to share information and knowledge about a topic. The idea is for everyone to be respectful, foster dialogue, and listen to each other. The purpose or outcome is to find common ground from which collaboration may occur or a decision can be made. Traditionally, circles are planned in advance and the forum convenes over several days. Contemporary listening circles use invitations and an agenda and are somewhat more formal, but they have the same purpose. Every attempt is made to follow the culturally appropriate protocol of the native community or group.

NLM listening circles seek to share information about NLM. This is accomplished by informing native leaders about outreach projects with tribal communities and promoting library science as a career path for Native American students. NLM also solicits input and feedback about how they can best disseminate health information and use technology, educate tribes about NLM and NIH funding opportunities, and identify collaboration and partnership opportunities.

The benefits of ensuring that senior decision makers receive and fully consider public input include:

- Making the NIH more transparent in its dissemination of information and education of the public.
- For the NIH leadership, increasing communication with the public by attending these forums, asking questions, listening, and stating what NIH can offer.
- For members of the public, learning how to access health information, leading to healthier communities and reduced health care costs to the nation.

Recommendation 6: Ensure that senior decision makers receive and fully consider public input.

COPR encourages NIH to develop a mechanism by which Institute and Center directors and senior NIH leadership can listen and receive public input in a way that is similar to the listening circle model used by the Native American population. If senior decision makers recognize and embrace the importance of public input, this will ensure that future NIH research and priority-setting decisions are transparent and public input is solicited.

7. Fully Utilize Advisory Councils and Their Public Members

Each Institute and Center charter mandates that persons who represent the general public fill a certain number of advisory council seats. The 1998 IoM report noted that slots reserved for public members on some advisory councils did not always represent the broad range of NIH public constituencies. According to the report, underrepresented groups included representatives of patients, their families, and persons with special health problems (e.g., particular ethnic groups, low-income populations, and women).

In contrast, the NCCAM charter requires that three of six advisory council slots reserved for public members be filled with persons who represent the interests of individual consumers of complementary and alternative medicine. While not necessarily endorsing a rigid mandate for all Institutes and Centers, COPR supports the inclusive intent that this requirement represents.

Beyond the selection of advisory council public members is the issue of identifying those members as such. There are a variety of cultures and practices among the Institutes and Centers in this regard. Some Institutes and Centers, perhaps to make sure that all advisory council members are seen and treated equally, do not identify individuals who fill their public slots. This interest in equality is legitimate, but care should be taken that this practice does not obscure the fact that some public slots may be filled by persons who do not represent what most would consider the general public. It is therefore essential that each Institute and Center examine its selection and identification of advisory council public members. Institutes and Centers should fill those slots with individuals who represent the sensibilities of a broad range of public constituencies, and consider identifying them so they might be fully utilized as valuable sources of public input.

Further, the National Research Council's 2003 report, *Enhancing the Vitality of the National Institutes of Health: Organizational Change to Meet New Challenges*,[3] noted that "Advisory councils should routinely and consistently be consulted in the priority-setting and

planning process of an institute." COPR agrees that advisory councils and their public members should be recognized as an invaluable source of and conduit for public input into the Institute and Center priority-setting process.

Examples of this principle are found in the recent practices of the National Institute on Deafness and other Communication Disorders (NIDCD) and NINDS. Public members of the NIDCD advisory council served on the subcommittee charged with writing the NIDCD Strategic Plan. The day before the group began writing, public members from many organizations (from small self-help and advocacy groups to large medical, scientific, and allied health organizations that certify medical and allied professional programs) provided written or oral testimony about research needs. Their information and perspectives influenced the content of the strategic plan. NINDS also involves advisory council public members in several important subcommittees devoted to key planning and priority-setting issues, including the subcommittee charged with developing the NINDS Strategic Plan.

NCCAM also uses its advisory council as a conduit for public input. NCCAM advisory council meetings include open forums where members of the public offer input. This is an important, regular access point for the public to communicate with senior decision makers. COPR strongly urges that each Institute and Center consider implementing similar practices.

The COPR urges Institutes and Centers to enhance the role of public members on Institute and Center advisory councils and consider creating new mechanisms to empower them. One possibility would be to bring the public members together regularly to discuss their role as public members, further define their role and how they bring the public perspective to council efforts, and work to identify new ways they can work together to enhance their participation on NIH Institute and Center councils. Another possibility would be to examine the role of Institute and Center Offices of Public Liaison in orienting public members of NIH advisory councils.

The benefits of ensuring that advisory council public members represent the public and that advisory councils are used as a resource and conduit for public input include:

- Offering a permanent, easily identifiable, accessible avenue for public input at the highest levels of Institutes and Centers.
- Demonstrating to the public that their viewpoints and input are valued and taken seriously.
- Providing an opportunity for open communication and building public member partnerships across the NIH at the highest levels.

Recommendation 7: Ensure that advisory council public members represent a broad range of public constituencies and that the councils are fully used as an important avenue for public input.

NIH and the Institutes and Centers should ensure that public advisory council members represent a broad range of public constituencies. Consideration should be given to the advantages of enhancing transparency and effectiveness in identifying the public members as such. Steps should be taken to ensure that public advisory council members are fully used as an important source of public input into the research priority-setting process through involvement in activities such as strategic planning. Finally, public advisory council members should be thoroughly oriented and empowered in

their role as representatives of the public perspective through training and cross-Institute meetings.

Transparency

In considering public input into the research priority-setting process at NIH, one issue warrants special focus—transparency. Transparency refers to how clear, understandable, and accessible to the public is the process by which NIH sets research priorities, and how much of the process is open to public examination.

In an age of diminishing budgets, pressure for information about how research dollars are spent and how such decisions are made will only increase. One of three themes of the NIH Roadmap is Re-engineering the Clinical Research Enterprise. This involves, in part, engaging the public more fully in the clinical research enterprise, which requires the public's trust. Transparency is an essential ingredient in developing and maintaining public trust.

To study the transparency of the NIH research priority-setting process, COPR examined the activities and practices of various Institutes and Centers. For a perspective external to NIH, interviews were conducted with Voluntary Health Association (VHA) workers. COPR members asked questions about VHA experience in working with NIH, and solicited input on transparency and suggestions for improvement.

In general, the results of these interviews were consistent with and significantly confirmed major conclusions and observations reached as a result of COPR's other investigations. In particular, the suggestion that NIH leadership leave the campus to meet the public through town meetings and regional outreach activities received nearly universal support. Details of the interviews are not enumerated here, but the information obtained is incorporated throughout this report.

Enhancing Transparency at NIH

In considering the goal of enhancing transparency, three types of activities were identified as fundamentally important: education, access, and active listening. Education involves teaching the public about how the priority-setting process works and about what is being done to obtain and use public input. Access relates to the availability of ways public input can be given that will influence research priority setting. This includes the public's ability to observe the process and to see how decisions are made and what factors are considered.

It is not enough that transparency exists in the system in the form of opportunities for education and points of access. Active listening relates to the need to solicit and understand the public's view of the NIH research priority-setting process. This perspective is essential; the perception of a lack of transparency is just as damaging to public trust as an actual lack of transparency. Some representative examples of these three activities are discussed in the following paragraphs. There is some overlap in the activities discussed here and those mentioned in the previous section because best practices for public input also enhance transparency.

8. Educate the Public about the NIH

The National Institute of Child Health and Human Development (NICHD) NIH 101 program helps educate the public about the NIH. The program is a seminar conducted as requested by the NICHD Program and OPL staff. Subjects include presentations from the Institute and Center Office of Budget and Communications and an optional tour of one of the NICHD intramural labs on the NIH Bethesda campus.

The NIH 101 presentation includes a brief history of NIH and NICHD, information about how budgets and research priorities are set, a breakdown of the grant review process, information about how NICHD communicates research findings, and instructions for finding other useful NIH information.

The program is offered to non-NIH research advocacy or patient representative organizations. NICHD publicizes this offering through Friends of the NICHD and other contacts. Typically, new staff members of research or patient advocacy groups who need to understand NIH inner workings take advantage of the program, but other groups can request a presentation.

This NIH 101 was developed several years ago, after the IoM report's release, as an effort to engage interested groups. The presentation is tailored to audience interests and generally is well received.

NICHD should be applauded for its innovation and initiative in engaging groups and responding to requests for information. The NIH 101 class has worked well for NICHD, but more could be done to promote the program's availability. A Web-based version has yet to be developed and most seminars take place on the NIH campus rather than around the country. Still, this program is a solid example to be replicated by other Institutes and Centers in their efforts to engage the public and enhance the transparency of their work.

The benefits of developing educational tools and materials for the public include:

- Helping the public better understand the multiple factors and complexity involved in the research priority-setting process.
- Informing the public about efforts already being made by the Institutes and Centers to solicit and incorporate public input.
- Helping the public become more effective collaborators with the Institutes and Centers in the research priority-setting process.

Recommendation 8: Actively develop tools and materials that help educate the public about the research priority-setting process and opportunities for public input.

One tool might be a series of Web-based interactive training modules or tutorials. More specifically, it is recommended that two of these modules focus on 1) basic information on how the research priority-setting process works at NIH, and 2) basic information on accessing the NIH and providing input. While these topics are the focus of this report, it should be noted that the concept of using this medium need not be confined to these topics, and tutorials on a variety of subjects would be useful. These subjects could include basic information about the NIH, the grant process, the NIH Roadmap, and others. The advantages of this approach are many. The

tutorials are self-paced and individuals can review and refer to them as often as needed. It is possible to use a broad range of media, including other Web pages, video, text, and sound. New information and changing data, such as budget numbers, can easily be incorporated.

9. Enhance Access

One transparency-enhancing activity that improves public access is the NCCAM Stakeholder Forums. In developing their second 5-year strategic plan, NCCAM held two open forums, one on the NIH Bethesda campus and one in Seattle, Washington. People and organizations on NCCAM's mail and e-mail lists were invited to participate. The forums were publicized through the media and on the NCCAM Web site.

During the forums, participants testified about their experiences and opinions related to development of the NCCAM Strategic Plan. A listening panel of top NCCAM administrators, including the NCCAM Director, heard the testimony. During this process there was a brief opportunity for questions and answers and dialogue among participants and the listening panel. At the end of each forum, the Director summarized major themes and offered initial thoughts and responses. After the forums, information obtained was transcribed and incorporated into the strategic planning process. A draft plan will be posted on the NCCAM Web site and the public will have another opportunity to comment.

The NCCAM Stakeholder Forums are notable in several ways for their impact on enhancing transparency. They solicit public input from the beginning of the strategic planning process and address issues of education and access. By providing an opportunity for dialogue with and response by the listening panel, members of the public in attendance received immediate information and feedback on their input.

The forums also provided a venue for publicizing other opportunities for public input, such as the ongoing acceptance of written public comments and the opportunity for online public comment on the draft strategic plan. Holding one of the forums off campus to increase accessibility was commendable. COPR feels that such stakeholder forums are worthy of consideration by all Institutes and Centers in developing their strategic plans.

Another example of improved access and enhanced transparency is the www.getinvolved.nih.gov Web site. The Director's OPL, Institute and Center OPLs, and Communication Directors have undertaken an extensive effort to improve the Web interface and make information more accessible about resources and opportunities for public input and participation. *The NIH Public Bulletin* is a product of NIH-wide information sharing and coordination, and provides a public resource that no single Institute or Center could offer.

Every month, NIH Institutes and Centers contribute information about public outreach events, public resources, and special public announcements. The Office of the Director's OPL compiles the submissions and posts them in the *NIH Public Bulletin* on the www.getinvolved.nih.gov Web site. The Institutes and Centers can also post items for public input or comment in this central location.

This is an ongoing process, and the work of continually improving access for members of the public to learn about input opportunities and provide input should be maintained and supported.

The benefits of enhancing access to the research priority-setting process include:

- Allowing the Institutes and Centers to obtain input from a broader cross-section of the various NIH publics.
- Communicating to the public that its opinions are valued, sought after, and used as an integral part of the research priority-setting process.
- Building a sense of inclusion and providing opportunities for involvement among the various NIH publics.

> **Recommendation 9: Continue to search for mechanisms that encourage public input into the research priority-setting process and that are easily accessible and provide information-sharing opportunities.**
>
> **Seeking ways to promote and facilitate public input into the research priority-setting process should be an ongoing agenda item for all Institutes and Centers. In addition to constantly attempting to find new avenues for public access to the process, existing methods should also be examined, evaluated, and refined.**

10. Practice Active Listening

A transparency-enhancing activity related to the concept of active listening is the 2003 Survey of Cancer Advocacy Organizations conducted on behalf of NCI and the NCI Director's Consumer Liaison Group (DCLG). NCI contracted with an independent market research firm to conduct a survey of 152 cancer advocacy organizations.

The survey solicited information about each organization and its familiarity with NCI, thoughts about future DCLG direction and activities, and preferred communication methods. The survey yielded important information about respondents' perceptions of and experiences with NCI. Respondents discussed NCI efforts that were useful and effective and those that needed improvement.

Although NCI transparency and research priority setting were not the survey's main purpose, some information was directly relevant. An example is a recommendation that NCI find better ways to communicate to the cancer advocacy community its strategy and how it prioritizes research.

The survey was particularly laudable in its use of an approach that actively and thoroughly solicited information from this sector of the public. Such proactive measures for obtaining public input are worthy of serious consideration and replication across Institutes and Centers.

The benefits of actively soliciting information from constituents and the public include:

- Obtaining important information from constituent public members that otherwise would be missed.
- Using the information regarding experiences and perceptions to enhance and improve the transparency of the process.

- Improving the perception of NIH and the individual Institutes and Centers regarding their commitment to and interest in a transparent process.
- Creating trust by soliciting and valuing public comments.

Recommendation 10: Actively solicit information from constituents and the general public about the public's experiences and perceptions of transparency at NIH.

It is not enough to passively await comments from the public regarding their experiences and perceptions of transparency at NIH. The Institutes and Centers should actively seek this input on an ongoing basis. If the costs involved are prohibitive, this may be an opportunity for a number of Institutes and Centers to pool their resources.

Support and Funding

11. Provide Adequate Resources

Implementing some of the above recommendations will require a significant investment of funding, staffing, and support. In a period of declining budgets, this may involve difficult choices. Nevertheless, COPR believes that the goals of improving public input into the research priority-setting process and enhancing the transparency of that process are extremely important, particularly when these goals are considered in light of their impact on increasing public trust.

Recommendation 11. Provide adequate resources in terms of funding, support, and staffing to allow for the successful accomplishment of these recommendations.

Whether support is provided by increasing personnel and other resources within NIH, providing a budget to contract services from outside vendors or other agencies, or improving resource sharing among Institutes and Centers, the commitment to improving public input and enhancing transparency must be serious. Implementation of these recommendations will only be effective if the level of support is adequate to the tasks involved.

Conclusions

The guarantee of public input and participation in the NIH research priority-setting process and the transparency of that process are essential to promoting public trust in the research enterprise. This report identifies seven principles for improving public input into the research priority-setting process, and offers examples of best practices that effectively employ these principles and recommendations related to each. The report also cites three activities with specific examples that enhance transparency, and makes accompanying recommendations. Finally, the report addresses the need for adequate funding and support for implementing the recommendations.

The COPR recognizes that significant opportunities exist for public input and transparency that have not been identified in this report. We urge senior staff at each Institute and Center to consider not only the *process* for public input, but also the *intent* of each example cited. The spirit in which this report is received will determine the effectiveness of the efforts of each Institute and Center in this regard.

Support for and belief in the importance of public input into the research priority-setting process at NIH must be embraced from the top down. The degree to which this occurs will determine the success of attempts to incorporate public input, how transparent the process is, and ultimately how trustworthy the NIH, the Institutes and Centers, and the clinical research enterprise are in the public's mind.

It is clear from our efforts that many Institutes and Centers at the NIH take the issue of public input and transparency seriously, but more could be done. The NIH has shown genuine concern for issues of public input and transparency; however, more coordination of activities and cooperation between the Institutes and Centers would be beneficial. NIH efforts related to public input and transparency are improving substantially, but this is clearly an endeavor for which there is no end.

Appendix

Members of the Director's Council of Public Representatives
April 1, 2003 – March 31, 2004

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Acronyms

COPR	NIH Director's Council of Public Representatives
DCLG	NCI Director's Consumer Liaison Group
ICs	NIH Institutes and Centers
IoM	Institute of Medicine
NIH	National Institutes of Health
NCCAM	National Center for Complementary and Alternative Medicine
NCI	National Cancer Institute
NIAAA	National Institute on Alcohol Abuse and Alcoholism
NIAMS	National Institute of Arthritis and Musculoskeletal and Skin Diseases
NICHD	National Institute of Child Health and Human Development
NIDCD	National Institute on Deafness and other Communication Disorders
NIDDK	National Institute of Diabetes and Digestive and Kidney Diseases
NIEHS	National Institute of Environmental Health Sciences
NIMH	National Institute of Mental Health
NINDS	National Institute on Neurological Diseases and Stroke
NKDEP	National Kidney Disease Education Program
NLM	National Library of Medicine
OPL	Officer of Public Liaison
PIO	Patient Interest Organization
PIPWG	Public Input and Participation Work Group
VHA	Voluntary Health Association