



**Testimony  
Before the Select Committee on Homeland  
Security  
United States House of Representatives**

**The NIH Biomedical Research  
Response to the Threat of  
Bioterrorism**

*Statement of*

**Anthony S. Fauci, M.D.**

*Director*

*National Institute of Allergy and Infectious  
Diseases*

*National Institutes of Health*

*Department of Health and Human Services*



**For Release on Delivery  
Expected at 1:00 PM  
on Thursday, June 3, 2004**

Mr. Chairman and Members of the Committee, thank you for the opportunity to speak with you today about the role of the National Institutes of Health (NIH) in the execution of our national biodefense research strategy.

The destruction of the World Trade Center, the attacks on the Pentagon and an airliner over Pennsylvania, and the anthrax attacks in the fall of 2001 clearly exposed the vulnerability of the United States to acts of terrorism. In particular, the anthrax attacks made it very clear that the possibility of the use by terrorists of deadly pathogens or biological toxins such as those that cause anthrax, smallpox or botulism represents a serious threat to our Nation and the world. The Administration and Congress responded aggressively to this threat by significantly increasing funding for biodefense preparedness and research.

The National Institute of Allergy and Infectious Diseases (NIAID) is a component of NIH and a leading federal agency for biomedical research concerning potential agents of bioterrorism that directly affect human health. NIH, and particularly NIAID, has devoted the increased biodefense research funding to an aggressive, broadly based research program designed to provide the American people with medical countermeasures, i.e. vaccines, therapeutics, and diagnostics against a range of bioterrorist threats. My remarks today will specifically address three aspects of our biodefense research activities. I will first describe the NIH biodefense research program, including a few examples of our recent accomplishments. Next, I will summarize how NIH biodefense research is coordinated with research carried out by other Federal agencies. I will close by discussing how NIH is informed about and responds to new bioterror threats that might arise.

## **NIH Biodefense Research**

The NIH research agenda for defense against bioterrorism was developed through a comprehensive and systematic strategic planning process. In February 2002, we convened the Blue Ribbon Panel on Bioterrorism and Its Implications for Biomedical Research, with membership composed of distinguished researchers from academic centers, private industry, government civilian agencies, and the military. Three key documents were developed based on this panel's advice and on extensive discussions with other Federal agencies: the *NIAID Strategic Plan for Biodefense Research*, the *NIAID Research Agenda for CDC Category A Agents* (for those agents that pose the gravest threat), and the *NIAID Research Agenda for CDC Category B and C Agents* (agents whose biological properties make them more difficult to deploy or less likely to cause widespread harm). The Strategic Plan provides a blueprint for the conduct of basic research on microbes and host immune defenses, as well as targeted, milestone-driven development of drugs, vaccines, diagnostics and other interventions that would be needed in the event of a bioterror attack. The two biodefense research agendas describe short-term, intermediate, and long-term goals for research on the wide variety of agents that could be used to conduct such an attack.

NIH has moved rapidly to execute its biodefense strategic plan and significant progress toward reaching many of the goals set forth in the research agendas has already been made, as described in two recent progress reports. With regard to basic research, which is needed to understand more about how pathogens interact with human hosts, NIAID-supported researchers and their international colleagues have completely sequenced the genomes of representative bacteria considered to be bioterror threats, including multiple strains of the anthrax bacterium, as well as at least one strain of every

potential viral and protozoan bioterror pathogen. Another NIAID program is supporting studies of the human innate immune system, which is comprised of broadly active “first responder” cells and other non-specific mechanisms that are the first line of defense against infection. The development of methods to boost innate immune responses could lead to fast-acting countermeasures to mitigate the effects of a wide variety of bioterror pathogens or toxins; in addition, manipulation of the innate immune system could lead the way towards the development of powerful adjuvants that can be used to increase the potency and effectiveness of vaccines.

NIH also has moved aggressively to expand national biodefense research capabilities by investing in several research infrastructure development programs, including manpower and facilities. For example, NIAID recently funded eight Regional Centers of Excellence for Biodefense and Emerging Infectious Diseases Research. This nationwide network of multidisciplinary academic centers will conduct wide-ranging research on infectious diseases that could be used in bioterrorism, and will develop diagnostics, therapeutics and vaccines needed for biodefense. In addition, these Centers will serve as the training ground for future generations of biodefense researchers. The Centers also partner with State and local public health agencies to ensure the strongest coordinated response to a bioterrorist event. In addition, NIAID is supporting the construction of two National Biocontainment Laboratories (NBLs), which will include facilities built to Biosafety Level 4 standards and will therefore be capable of safely containing any pathogen, as well as nine Regional Biocontainment Laboratories (RBLs) with Biosafety Level 3 facilities. These high-level research laboratories will provide the secure space needed to carry out the nation’s expanded biodefense research program in a setting of safety for both biodefense workers and the surrounding community. Other ongoing projects will expand intramural facilities at Bethesda and

Rockville, Maryland, at our Rocky Mountain Laboratories in Hamilton, Montana, and in Frederick, Maryland.

The ultimate goal of all NIH biodefense research is the creation of new and effective medical countermeasures, including vaccines, therapeutics, and diagnostics against potential bioterror agents. Substantial progress toward this goal has already been achieved. In the area of therapeutics, for example, NIAID-supported scientists have identified a drug that may prove useful in treating both smallpox and the complications of smallpox vaccination. This agent, called *cidofovir*, is approved by the FDA for treating viral eye infections in HIV-infected patients. NIAID studies also are investigating the use of antibodies that can bind to and block the action of toxins produced by the anthrax bacterium, as well as botulinum toxin.

New and improved strategies for the development of vaccines against smallpox, anthrax and other potential bioterror agents are being vigorously pursued, with the objective of adding them to the Strategic National Stockpile (SNS) as quickly as possible. For example, NIAID is supporting and overseeing the rapid development of the next-generation anthrax vaccine known as recombinant protective antigen, or rPA. Clinical trials of rPA are ongoing; results to date build on findings in animal studies that suggest the vaccine is safe and capable of evoking a robust immune response. Researchers also will test, in animals, whether protection against anthrax can be enhanced by receiving the rPA vaccine in addition to antibiotic therapy following exposure to anthrax spores. This development effort is on track for Project BioShield to award contracts this year to achieve the goal of adding 75 million doses of rPA vaccine to the SNS to protect 25 million U.S. citizens.

NIAID-supported researchers also are testing several new smallpox vaccines that may prove at least as effective as the current smallpox vaccine, and can be used by a broader population, including those who are immunocompromised. One of these, modified vaccinia Ankara (MVA), is based on a strain of the vaccinia virus that replicates less robustly than the traditional Dryvax vaccinia virus, and which is also known to cause fewer side effects. Human trials of MVA vaccines are under way at NIH and elsewhere. Encouragingly, recent studies by NIAID intramural scientists and their colleagues have shown that MVA protects monkeys and mice from smallpox-like viruses. NIH also has launched the first human trial of a vaccine designed to prevent infection with Ebola virus. The trial vaccine is made from parts of the viral DNA, and is similar in design to other investigational vaccines that hold promise for controlling such diseases as AIDS, SARS, and infection with West Nile virus.

### **Coordination of Biodefense Research**

Although NIH is a leading agency in government-sponsored biomedical biodefense research on agents that directly affect humans, it is by no means the only agency involved; the Centers for Disease Control and Prevention (CDC), the Food and Drug Administration (FDA), the Department of Defense (DoD), the Department of Homeland Security (DHS), the Department of Agriculture (USDA), and other governmental organizations also play important roles. Coordination among the various agencies involved is therefore extremely important. In broad terms, the NIH biodefense research agenda and activities are coordinated at three distinct levels: within NIH, within DHHS, and across the government as a whole.

Within NIH, NIAID is responsible for the bulk of NIH-sponsored biodefense research;

other NIH institutes, however, also make significant contributions. The focal point for trans-NIH coordination and planning of biodefense activities is the NIH Biodefense Research Coordinating Committee. I am Chairman of this committee, which meets at least quarterly or more often, as needed. It is administered by the NIAID Office of Biodefense Research, which also serves as liaison office for NIH contacts with other federal agencies such as DoD and DHS regarding biodefense research and response.

At the level of the Department of Health and Human Services (DHHS), coordination of biodefense research between the CDC, NIH, FDA and other agencies within DHHS is the responsibility of the DHHS Office of the Assistant Secretary for Public Health Emergency Preparedness (ASPHEP). The ASPHEP Office of Research and Development Coordination holds periodic meetings with all governmental stakeholders in the development of medical countermeasures.

Members of my staff also meet regularly with the research community at Fort Detrick and the U.S. Army Medical Research and Material Command. Through such meetings, synergy in research and mutual support leading to the development of new drugs, vaccines, and diagnostic tests for the nation are achieved. My staff also holds meetings periodically with the Defense Threat Reduction Agency and the Defense Advanced Research Projects Agency, two important entities within the research infrastructure in the DoD.

At the highest level, coordination of biodefense research is carried out by the White House, and in particular the Homeland Security Council, now led by Frances Townsend, and the National Security Council. In addition, the Committee on Homeland and National Security of the National Science and Technology Advisory Council also

participates, especially through its Weapons of Mass Destruction Medical Countermeasures Subcommittee.

Although these three levels describe the basic structure through which the Nation's biodefense research programs are formally coordinated, NIH, as the lead biodefense research agency, collaborates daily with the other federal agencies and is party to a large number of interagency programs, informal contacts, and communication mechanisms that significantly contribute to the efficiency and effectiveness with which biodefense research is carried out across the U.S. government.

### **Threat Assessment and the Research Response**

We developed the *NIAID Strategic Plan for Biodefense Research* and the two research agendas based on an overall threat assessment formulated by CDC in close cooperation with the intelligence community. Category A agents are the most dangerous microbes and toxins; these include anthrax, smallpox, plague, botulism, tularemia, and hemorrhagic fevers caused by viruses such as Ebola. These agents were given the highest priority because they a) are relatively easily disseminated or transmitted from person to person; b) result in high mortality rates with the potential for major public health impact; c) would likely cause significant social disruption; and d) require special action for public health preparedness. Category B agents are in the second tier of priority. They are agents that a) are moderately easy to disseminate, b) result in moderate morbidity and low mortality rate, and c) require specific enhancements of national diagnostic capacity and disease surveillance systems. Category C Agents have the next highest priority. They include emerging pathogens that could be engineered for mass dissemination in the future because of their

availability, ease of production and dissemination, and potential for high morbidity and mortality rates and major health impact.

To receive information about new threats that may arise, we work closely with DHS, which provides threat assessments concerning issues germane to our research. Because new infectious disease challenges emerge naturally on a regular basis, NIH has considerable experience in rapidly mobilizing research resources to confront new infectious disease threats. This experience serves us well when called upon to adjust our research priorities in response to new intelligence information.

In closing, I am confident that the biomedical research agenda we have formulated concerning potential agents of bioterrorism is well conceived, and will rapidly lead to new and improved medical countermeasures against agents of bioterrorism. I am also very pleased with the degree of coordination and cooperation between NIH and other federal agencies involved in carrying out biodefense research.

I appreciate this opportunity to testify before you today, and I would be pleased to answer any questions that you may have.