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Mr. Chairman and Members of the Committee:

Thank you for the opportunity to testify before your committee on "Biological Warfare Defense Vaccine Research and Development Programs." I will review the status of vaccine development as well as the technological, financial, regulatory, and other challenges facing us. One of the key points I will address today is that it is just as **important to pursue other biomedical and biotherapeutic solutions** – in addition to vaccines – that can protect the American people from a variety of pathogens and an increasingly sophisticated enemy. The biotechnology industry is engaged in a tremendous research and development effort dedicated to that end.

Another key point I will discuss today is the **critical importance of government support of this industry,** largely made up of small companies that have no base of marketed products and profits to fund research.

I am here today representing BIO, the Biotechnology Industry Organization, whose role in American health care, safety and security is becoming more apparent every day.

BIO represents more than 1,000 biotechnology companies, academic institutions, state biotechnology centers and related organizations in all 50 U.S. states and 33 other nations. BIO members are involved in the research and development of health care, agricultural, industrial and environmental biotechnology products, and have recently formed a special biodefense taskforce, of which I am a member.

I also am president and CEO of EluSys Therapeutics, Inc. and before that, held leadership positions with a global pharmaceutical manufacturer for almost 25 years. My company and the biotechnology industry are engaged in cutting edge science that is uniquely poised to benefit Americans in many ways.

Biotechnology is one of the most research-intensive industries in the world. The US biotech industry spent $10.7 billion on research and development in 2000. And in many cases, the benefits already have been realized. Close to 120 FDA-approved biotechnology drug products and vaccines have helped some 250 million people worldwide. *Seventy-five percent of these medicines were approved in the past six years*. Clearly, biotechnology endeavors have made a significant contribution to the nation's health.

EluSys is an example of how the biotech industry works – we are a fledgling company that has licensed early-stage technology from academia, and are engaged in the process of developing safe, effective and marketable therapeutics for a range of unmet medical needs. Since the company's founding in 1998 we have worked collaboratively, first with the Defense Advanced Research Projects Agency and now the US Army Medical Research Institute of Infectious Diseases, to pursue development of our unique therapy against potential biological weapons of mass destruction, such as anthrax, hemorrhagic fever, Ebola virus, plague and smallpox.

Therapeutic Options

I'd like to take a moment to explain the therapeutic options our nation needs to explore and understand in connection with biological weapons and the diseases they cause. There are actually three levels of treatment:

· Prophylaxis – that is, vaccines to prevent people from becoming ill before they are exposed

· Antibiotics and antivirals that need to be given immediately upon diagnosis or exposure, and

· Antidotes and other therapeutics that can cleanse the blood of toxins once the disease has developed.

Each of these approaches is critical, and all are necessary. None of them is at a stage where we can rest

easy.

Let me explore each of these treatment options in a little more depth so you can better understand how they differ and how they complement each other.

Vaccines

We now have two vaccines for biowarfare pathogens, one for anthrax and one for smallpox, and there are others in development. The major challenges in vaccine technology are to improve their safety, to develop vaccines against a variety of pathogens and strains, to shorten the time needed to inoculate people, to be able to mass produce them and to determine how and when – and to whom – to administer them.

Those are the major technical challenges. There are, however, added considerations, especially in regard to military vs. civilian populations. The current anthrax vaccine requires six injections over 18 months, plus booster shots, to provide full immunity. While this may be conceivable in the military population, it is clearly unrealistic in the civilian population. If you think of the logistics of tracking a five or six vaccination series and boosters for 280 million people for anthrax, you can easily visualize problems. Potential improvements in vaccine regimens notwithstanding, it is important to have post-exposure options for both the military and civilian populations. Therapeutics that can be administered post-exposure are obviously more desirable for civilians, but also necessary for the military.

Antibiotics and Antivirals

We are fortunate to have effective antibiotics already available. In addition, there are antiviral therapies currently available, for CMV (cytomegalovirus), HIV/AIDS and herpes that may or may not be effective against viruses used in biowarfare. Biotechnology and pharmaceutical companies are working to find new antibiotic and antiviral alternatives in anticipation of the development of strains that are resistant to available drugs.

Antibiotics inactivate or kill bacteria, including anthrax. But they don't protect against viruses, and they can't help someone who is experiencing signs and symptoms of anthrax or another such disease. If the bacteria already have released toxins into the bloodstream, there is nothing that can be done. It is too late. That is where blood-cleansing technologies like the one EluSys is working on comes in.

Therapeutics and Antidotes

The unique Heteropolymer System that EluSys is developing uses two monoclonal antibodies chemically joined together, like biological double-sided tape. One of these antibodies sticks to the target to be removed (such as the anthrax toxin); the other binds to a receptor found on human red blood cells. The red blood cells then carry the pathogen to the liver for destruction and return unharmed to the normal blood circulation. This whole process happens within minutes.

Preliminary test results show that the EluSys Heteropolymer (or HP) System works like a temporary vaccine in that it confers an instant but temporary state of immunity to pathogens that are carried in the bloodstream, whether bacterial or viral.

Unlike vaccines, antibiotics and antivirals, the HP System can be engineered to be active against anything that circulates in the bloodstream – bacteria, toxins and viruses.

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Post-Exposure Options

For the general public, we need more post-exposure options; it is not feasible or practical to vaccinate the entire population against anthrax or other toxins. There are side effects to these vaccines and the benefits probably would not outweigh the risks.

Nor would antibiotics protect people from a possible scenario in which a pathogen is aerosolized and released through a building's air system. Many people could be exposed and infected without knowing it – there would be no telltale white powder. A few days later, when they started experiencing symptoms and went to their doctors, it would be too late.

Inhalation anthrax involves exposure to anthrax spores that enter cells in the lung and become the bacteria that can produce toxin. The toxin and the bacteria then enter the bloodstream and circulate through the body.

While antibiotics are effective against the bacteria, they are not effective against the toxin, and it is the toxin that can rapidly cause widespread inflammation and cell death in multiple organs, including the brain, liver and spleen. Once the toxin has been released into the bloodstream and symptoms have appeared, there is no evidence that a course of antibiotics will be effective in preventing death.

The EluSys HP System, by removing the toxin from the bloodstream, has the potential to fill an unmet need in the armamentarium against anthrax. Since it works against the toxin, it may buy crucial time to allow for later-stage antibiotic treatment.

The Need for Government Support

EluSys is one of almost two dozen American companies involved in the development of biodefense vaccines and therapeutics. These companies are addressing a range of products we fervently hope we never need. Avant, in Massachusetts, is developing anthrax and cholera vaccines. Genelabs, in California, is working on broad-spectrum antimicrobial and antiviral drugs. Luminex, in Texas, is exploring biodetection systems. Inotek, in Ohio, is researching a product to fight inflammatory biowarfare agents such as plague and Ebola virus.

Companies in this field of research and development have identified barriers that hinder quick, large-scale development and production of these products. While there may not be unanimity about how to specifically overcome all these barriers, let me provide you with my view – a prevailing view among industry leaders.

EluSys and other biotechnology companies rely heavily on venture capital to support our work, and at this juncture, we need the support of government as well to move forward as quickly as the science allows.

The normal drug-marketing paradigm assumes that research will lead to drugs that can be marketed to certain patient populations. However, the market for agents against biological toxins is small and uncertain: there is no guarantee that the vaccines or therapeutics we develop will ever be needed. And since biowarfare is an area of national defense that may or may not have a market, support from the venture capital and financial sectors for this research is limited That is why we and other companies need the support of the government to continue to develop these important drugs and devices.

Aggressive government funding will enable companies like mine to significantly expedite the development of important agents against biological toxins. For example, in the normal course of development, it would take four to six years to get the EluSys antidote ready to treat human beings exposed to anthrax. Government funding of $50 million will enable us to develop the antidote in roughly two years.

Long-Term Financial and Administrative Considerations

In addition to supporting our research on an accelerated schedule, the government must provide support in the form of long-term contracts. This will help maintain consistent revenue streams so our companies can continue to draw support for both biodefense and commercial applications of our products and technologies.

The biotechnology industry also needs support from government that goes beyond funding and includes:

1. Protection from liability. We are in somewhat uncharted territory, dealing with toxins that pose significant danger of harming people. All drugs carry the potential for side effects – some serious, some not – and drugs to protect or counter the effects of these toxins may cause some negative side effects. However, in order to focus our efforts on developing agents for the broad population, the biotechnology industry needs government to indemnify our companies to protect them from lawsuits. In fact, fear of liability has prevented many companies from even considering the development of vaccines.

2. Support for security measures. The nature of our research – to develop protection for people against an agent that is deployed by those who want to harm people – puts our laboratories, researchers and other personnel at increased risk of danger. We ask the government to support the additional security measures we will need

3. FDA Support: In 1999, the Food and Drug Administration issued proposed regulations on efficacy data for products to prevent toxicity. In essence, the regulations would allow the data showing that the agent is effective against a certain biological, chemical, radiological or nuclear substance to be demonstrated through animal data only. The only human testing would involve the evaluation of safety to the drug without exposure to the noxious agent. FDA needs to adopt measures that will clarify and expedite the product review and approval process. This will help those of us who are pursuing countermeasures to toxins plan the further development of our agents.

In conclusion, I hope I have been able to give you a better understanding of the array of approaches we can bring to biodefense, and a sense of urgency about some broader issues of funding and administrative support. It is clear that use of biologicals in warfare and homeland terrorism is a great threat to Americans and the biotechnology industry stands ready to work side by side with the government to stamp out this threat.

Thank you for the opportunity to testify today. I'll be happy to answer any questions you may have.

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