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"One Year Later: Evaluating the Effectiveness of Project BioShield"

REGARDING THE IMPLEMENTATION OF PROJECT BIOSHIELD AND THE STATE OF BIODEFENSE IN THE UNITED STATES

Mr. Chairman, Ranking Member Waxman, members of the Committee, I am pleased to submit testimony to the Committee for the record regarding the implementation of the Project BioShield Act of 2004 and VaxGen's role in our nation's preparedness to respond to an anthrax attack.

Before turning to the substance of my testimony allow me to thank the Chairman, the Ranking Member and the rest of this Committee for your longstanding efforts to better protect this nation against the threat of bioterrorism. Your leadership is vital to safeguarding the American people.

My name is Lance Gordon; I'm President and CEO of VaxGen. I bring to VaxGen more than 20 years of experience in the vaccine industry, developing both vaccine products and companies. Prior to joining VaxGen, I served as CEO and a member of the Board of Directors of two vaccine companies, OraVax and North American Vaccines. While at OraVax, I developed and managed a 20-year program that supplied a 40-million dose stockpile of smallpox vaccine for the Centers for Disease Control. I am the inventor of ProHibit® for infant meningitis, the first bacterial conjugate vaccine to receive FDA approval, and led efforts that created a new vaccine licensed by the FDA for whooping cough. I was recently appointed to the National Vaccine Advisory Committee (NVAC). NVAC advises and makes recommendations to the Department of Health and Human Services (HHS) on issues related to preventing infectious diseases through immunization.

VaxGen, Inc. is a California-based biopharmaceutical company focused on the development, manufacture and commercialization of biologic products for the prevention and treatment of human infectious disease. Founded in 1995, one of VaxGen's business strategies emphasizes the development and commercialization of vaccines for the prevention of potential bioterrorism threats, specifically anthrax and smallpox.

VaxGen's Contract under Project BioShield

While we were disappointed in not having the opportunity to testify, it's only fitting for us to submit testimony to this Committee on the subject of Project BioShield since our

company was awarded the largest contract under the law to date. On November 4, 2004, VaxGen won a very competitive bidding process and was chosen by the Department of Health and Human Services to receive an $877.5 million contract to supply 75 million doses of a new generation, recombinant technology anthrax vaccine, known as recombinant Protective Antigen or rPA, for the nation's civilian Strategic National Stockpile. It was a competitive and open government procurement process in which any qualified bidder could participate and several did. The process conformed to all applicable rules and regulations. We believe the government chose us among several competing bids because of the advanced development status of our candidate vaccine, demonstrated manufacturing capacity and the proposal we submitted which was judged to be superior on technical, cost and scientific grounds.

Additionally, we believe that the government selected VaxGen because the BioShield program was enacted by Congress to speed the development of next generation medical counter-measures to terrorism. Existing anthrax vaccines have been around since the 1970s and are based on outdated technology. The nation required a safer, more effective, purer, next generation vaccine. Our technology is intended to meet those needs.

We are extremely proud to be developing the vaccine intended to protect millions of Americans in the event of an anthrax attack. This is a hugely important role and we're passionate about our commitment to assuring the public's safety and national security. We are pleased that Project BioShield allocated the funds that enabled us to win this contract to help our nation respond to the threat of bioterrorism.

The Project BioShield award we won last November followed on the heels of two competitive anthrax vaccine development contracts awarded to VaxGen by the NIH in successive years: an approximately $21 million development contract awarded in 2002 and an approximately $80 million development contract awarded in 2003. The decision to award each of the contracts to VaxGen included consideration of specific information on our candidate vaccine and our performance under preceding contracts. This history gave the government direct insight into the extent to which they could rely on our candidate vaccine and upon our ability to perform under the contracts. Avecia Group PLC of the United Kingdom won similar contracts from NIH to conduct parallel research on the anthrax vaccine, however their bid to supply vaccine to the Strategic National Stockpile under Project BioShield was rejected. Avecia initially protested their rejection but withdrew their protest. Although BioPort Corporation manufactures a previous generation, anthrax vaccine, known as AVA or Anthrax Vaccine Adsorbed, principally for the military, AVA did not meet the specifications contained in the HHS Request For Proposals (RFP-DHHS-ORDC-04-01) to supply a modern recombinant anthrax vaccine for civilian biodefense. To the best of our knowledge, BioPort has not developed such a candidate vaccine and did not bid on the HHS BioShield contract.

Reasons for Procuring New Anthrax Vaccine

Why did the U.S. government decide to procure a new anthrax vaccine? The answer is because the Institute of Medicine (IOM) recommended that that was the prudent thing to

do. In its 2002 report, "The Anthrax Vaccine: Is It Safe? Does It Work?," the IOM stated, "…the committee is convinced that relying on AVA [BioPort's vaccine] and the current specifications for its use is far from satisfactory. There is a need for research toward the development of a different and better anthrax vaccine, as well as a need for improvements in monitoring the safety of the current vaccine…. The committee concludes…that ***a new vaccine***, developed according to more modern principles of vaccinology, ***is urgently needed***."

VaxGen was extremely disappointed that BioPort misstated facts in its oral and written testimony before the Committee on July 14 and would like to set the record straight. While BioPort claims that VaxGen's vaccine will not have any clear advantages over their vaccine in terms of safety, efficacy, administration or production, nothing could be further from the truth. The following section of this testimony sets out the facts that debunk each of BioPort's erroneous statements.

Advantages of VaxGen's rPA102 Vaccine over BioPort's AVA Vaccine

VaxGen's vaccine, which we call rPA102, is intrinsically superior in terms of safety and consistency when compared with BioPort's AVA vaccine. VaxGen's rPA102 is composed of a single well defined protein, Protective Antigen, known to be the basis of protection against inhalation anthrax. BioPort's AVA is composed of filtered bacterial culture which, while including a variable amount of Protective Antigen, also includes an undefined mixture of other products of bacterial fermentation, as well as small amounts of anti-bacterial preservatives, formaldehyde and benzalkonium chloride. These extraneous components may account for the high rate of local (injection site) reactogenicity reported with AVA.

VaxGen's product specifications call for 95 percent purity. Modern recombinant technology has allowed VaxGen to consistently produce the vaccine at nearly 100 percent purity – significantly higher than what can be obtained using older technologies such as BioPort's. Published information on the BioPort vaccine reports variable protective antigen content of 30-65 percent with the remainder consisting of unspecified products of bacterial fermentation. In simple terms, BioPort's product is less consistent and has greater impurities than VaxGen's rPA102.

To date, VaxGen's anthrax vaccine is proving itself to have less reactogenicity. There have been no serious adverse safety events related to VaxGen's anthrax vaccine in either of our clinical trials. These trials included approximately 600 people and, the first of the two trials compared our vaccine with BioPort's AVA vaccine. The essential results of both of these trials were reviewed by the Office of Research and Development Coordination in the Office of Public Health and Emergency Preparedness at HHS, the National Institute for Allergy and Infectious Diseases and the FDA prior to the contract award. Although the Institute of Medicine found that BioPort's AVA vaccine is generally safe, the FDA in 2001 required that its packaging warn of potential side effects. They included lymphoma, lupus, seizures and death.

It is not surprising, then, that the July 7 issue of *Global Security Newswire* reported that although BioPort's vaccine has been offered to about 14,000 military personnel under a voluntary program that began on May 19 of this year, roughly 7,000 of them have refused to take it, despite a heightened risk of an anthrax attack on U.S. forces. The Global Security report further states that:

> *The high refusal rate comes amid persisting complaints by some service people and nongovernmental experts that the U.S. military has been reluctant to acknowledge a connection between the vaccine and uncommon but potentially debilitating side effects, which they say has hindered access to medical benefits and compensation. It comes also despite a determination in December by then-Deputy Defense Secretary Paul Wolfowitz citing classified intelligence, that there is "a significant potential for a military emergency involving a heightened risk" of an anthrax attack on U.S. forces.*

This refusal rate is astonishing in that it comes from a population that is well disciplined, trained to accept risks and is not known for bucking established procedures. In other words, if 50 percent of military personnel reject the existing vaccine out of safety concerns, it stands to reason that if that drug had been selected for use in the civilian stockpile the rejection rate would likely be higher still—and the nation's population as a whole would be unprotected. There should be little surprise that HHS sought a next generation, safer, purer, and more effective anthrax vaccine.

As for BioPort's claim that its vaccine is superior in terms of administration, that is patently untrue. VaxGen's vaccine requires significantly few doses for protection, and people receiving it will achieve the immune response in a far shorter time than they would with BioPort's vaccine. VaxGen's rPA102 has been designed from the start to provide long term protection following three doses administered over six months or less. BioPort's AVA vaccine, on the other hand, requires six doses administered over 18 months, plus annual booster doses. If you were contemplating getting shots, would you rather get three over six months or six over 18 months to be protected?

VaxGen's vaccine is being developed to protect before and after exposure to inhalation anthrax. BioPort's is only licensed for use <u>before</u> exposure. If the nation were attacked and did not have time to administer a vaccine for 18 months beforehand, as happened in the attacks on the capital in 2001, which vaccine would you rather have in the national stockpile: one that can protect you after the attack, or one that is useless unless given beforehand?

Furthermore, VaxGen's rPA102 is the first anthrax vaccine being developed specifically for prevention of inhalation anthrax, the most likely form of the disease to be used as a weapon of bioterrorism. AVA was developed for wool sorters disease, a skin infection occurring in textile workers handling raw wool containing anthrax spores. BioPort's

vaccine has never been formally tested and has no FDA approved label indications for use against inhalation anthrax. A sheep-based attack is not one of the 15 most likely and devastating threats we face according to the Department of Homeland Security's National Planning Scenarios; a biological attack using aerosolized anthrax is number two on that DHS list.

Finally, contrary to BioPort's assertions, VaxGen's vaccine wins hands down on cost. BioPort's vaccine costs $25.54 per dose. VaxGen's advanced vaccine costs only $11.70 per dose, or less than half the price of BioPort's. A full course of immunization (three doses) of VaxGen's vaccine costs $35.10, while a full course of AVA (six doses) costs $150, or more than four times as much. Further VaxGen's price is for vaccine supplied in syringes with safety needles included ready for use while the BioPort product is supplied in vials necessitating separate purchase of syringes and needles. Obviously, the savings can be spent on other, much-needed biodefense products. In other words, if HHS had bought the BioPort product, we would have been able to protect less than one quarter of the number of people we can with VaxGen's drug—or we would have had to spend at least four times as much, if not more.

BioPort's testimony before the committee sought to paint itself as the more experienced actor and VaxGen as new to the field. This portrayal is at best blatantly misleading. I have more than 20 years experience in successful vaccine development. Taken together, VaxGen's senior management team alone has over a hundred years experience in drug development, the bulk of that in vaccine development. VaxGen has been operating as an independent corporate entity since 1995 when it was spun out of Genentech, while BioPort was formed in 1998 to acquire of what had previously been the Michigan Biologics Institute.

BioPort itself has never developed a single product. It purchased an already existing product and a troubled manufacturing capability. It has long been known that the active component of AVA is Protective Antigen, and the technology for producing pure Protective Antigen is well established. The high rate of reactogenicity associated with AVA has been well known, at least since the Gulf War. Nevertheless, to our knowledge, BioPort has not made any meaningful attempts to improve the vaccine they acquired, nor to develop a modern, recombinant Protective Antigen.

BioPort itself has never taken a drug to FDA approval. More importantly, BioPort has no experience developing a product under the vastly expedited timelines required to meet the nation's biodefense needs. In fact, no other company has been through such a process; our product was the first truly new drug purchased under BioShield. Normal drug development is a difficult enough process, taking on average more than ten years and costing hundreds of millions of dollars. What we are working to achieve under BioShield with our new anthrax vaccine is to cut that timeline roughly in half. As a result, VaxGen is now one of the few companies that have actual, hands-on experience in BioShield drug development. We believe our expertise in this arena is a valuable asset both to VaxGen and to our country.

Advantages of a Single Supplier for Anthrax Vaccine

It is also important to address the issue BioPort raised that somehow the government's reliance on a single supplier to make the new generation anthrax vaccine is cause for concern. BioPort itself was for years the sole manufacturer of the anthrax vaccine and never appeared worried about that state of affairs. In fact, it not only defended its status extensively at the time on its own Web site, it continues to defend the practice on its Web site at least minimally today. Here is a question and answer from BioPort's Web site found on July 17, 2005:

> ***Why does our country have only one maker of this important vaccine?***
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> ***BioPort is not unique as the sole FDA-licensed manufacturer of a vaccine. There is currently only one commercial manufacturer for over half (17 of 31) FDA licensed vaccines in the US. There are currently NO manufacturers for 6 licensed vaccines and only 2 commercial manufacturers for 6 licensed vaccines. This is no coincidence. The US vaccine research and manufacturing industry is a complex and expensive business requiring years of effort and costing hundreds of millions of dollars to make these products available for protection of the public health and military. There is a limited potential for financial return of this investment. However, the personal rewards of helping produce a vaccine which can prevent disease, reduce suffering and save lives, is a strong motivating force for all of us at BioPort.***

But let's consider the single supplier issue on the merits, regardless of whether BioPort's position has changed. When VaxGen bid on the BioShield anthrax vaccine contract our proposal offered HHS the option that we would share our technology with another manufacturer to establish a second source of supply, an offer that still stands today. However, they asked that we delete that portion of our proposal, probably because of the following advantages of having a single supplier.

A single supplier not only saves money, it also eases distribution in the event of another anthrax attack. Just imagine the difficulties of trying to direct panicked civilians to choose among different anthrax vaccines in the middle of an attack when they live in the same city or live in different cities.

Having multiple companies simultaneously developing similar products for the same market also creates a strain on limited resources that could slow vaccine availability and jeopardize availability of countermeasures when needed. There is limited capacity for conducting critical and pivotal animal efficacy studies in compliance with the FDA's Good Laboratories Practice regulations as well as limited supplies of highly qualified clinical investigators and product liability coverage.

In conclusion, we'd like to emphasize to this Committee that Project BioShield is a valuable tool in helping our country defend itself against the threats posed by bioterrorists. VaxGen is proud to have received the very first contract under Project

BioShield and is honored to play a critical role in our nation's defense. Producing a vaccine that can save lives, reduce suffering and help protect our country from attack is a source of pride for our company. While our excellent partnership with the HHS, FDA and NIH is a first for our industry, we have made huge progress in a very short time and we are committed to produce and deliver a vaccine in almost half the time of the normal regulatory pathway, regardless of what challenges may lay ahead.