

“Taking Measure of Countermeasures (Part 1): A Review of Government and Industry Efforts to Protect the Homeland through Accelerated Research, Development and Acquisition of Chemical, Biological, Radiological and Nuclear Countermeasures”

Testimony of

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Good afternoon Mr. Chairman, Congresswoman Richardson, and Members of the Subcommittee. My name is John Clerici and I am a Principal of Tiber Creek Partners, a firm dedicated to assisting biotechnology companies throughout the world to ensure the development of the very best products that will have a positive impact upon public health and emerging infectious disease. For the last decade, my colleagues and I have had the opportunity to work with dozens of companies pursuing medical countermeasures targeting chemical, biological, radiological, and nuclear (CBRN) threats, many of which now sit in the U.S. Strategic National Stockpile and a number of which were deployed for use during the 2009 influenza pandemic. We have been involved in nearly every effort by the U.S. government to support and purchase these products over the last 12 years, including working with many of your colleagues in Congress to support legislation to protect the American people from a variety of public health threats. From this vantage point, I personally have seen both the good and the bad of this process

and I am delighted to share those observations with you in the hope that we can build upon the successes and learn from the challenges over the last decade with the mutual goal of ensuring our Nation is as prepared as possible.

I have three main observations that I would like to share with the Subcommittee this afternoon regarding the current efforts by what collectively is known as the “Public Health Emergency Medical Countermeasures Enterprise” in identifying and procuring medical countermeasures to address bioterrorism, nuclear preparedness, and emerging infectious disease.

First, the current laws passed during the last decade have proved generally satisfactory to provide the relevant public health officials with the legal authorities, funding and structure necessary to carry out their mission. However, the implementation of these laws has been unnecessarily burdened by constant internal and external reviews, delayed action, bureaucracy, and a lack of transparency. This has had a devastating effect upon the willingness of the private sector to participate in these programs. Second, there have been several recent instances, to include the ongoing crisis in Japan, where there has appeared to be a lack of proactive efforts to look toward an incident as a reminder that we need to bolster the knowledge base and understanding of what a mass casualty event in the U.S. would look like, and additionally demonstrates our need to more fully understand how medical countermeasures would be used, what resources are needed that are not currently available, and how best to reach those in need. I am concerned we are not being proactive to learn the proper lessons from these events. Last, there is a similar lack of proactive planning to address the sustainability of the medical countermeasures that have already been developed and purchased to maximize the value of the investments already made, as well to take full advantage of the benefits of sustainable, dual use, broad spectrum technologies.

With your permission, I would like discuss each of these observations in greater detail and offer some thoughts on proposed solutions that I believe are easily achievable in the short term.

As the Subcommittee is aware, in the last decade, Congress has passed several key pieces of legislation to address public health preparedness, including the Bioterrorism Act of 2002, the Project BioShield Act of 2004, the PREP Act of 2005, and the Pandemic and All Hazards Preparedness Act of 2006 (known as PAHPA). In addition, Congress has provided billions in Appropriations to support these programs. The PAHPA legislation, which created the Biomedical Advanced Research and Development Authority (BARDA), was meant to fill in the gaps in Project BioShield to help companies through the “valley of death” between advanced development and FDA approval, as well as streamline the procurement process. This bipartisan legislation, which earned the unanimous support of the House and Senate, was carefully crafted to provide the Executive Branch all the authorities needed to carry out this important public health mission. I do not think it can be disputed that PAHPA achieved its goal of providing BARDA with the toolbox it needs to do its job. Thus PAHPA should be reauthorized by Congress this year without the need for significant modification.

However, what was not anticipated by Congress in passing PAHPA, and what requires immediate attention, is the reality that the toolbox Congress provided to BARDA has been locked away while the organization is subjected to persistent internal and external reviews, as well as constant shifts in strategic direction, that have left industry confused and disheartened. Following what was generally viewed by the informed public health community as a very successful response to the 2009 influenza pandemic, BARDA underwent no less than three internal and external reviews during the course of 2010 to analyze its effectiveness. These

reviews resulted in a near stand-still of activity for almost a year and culminated in yet another shift in priorities for the organization. This included a transfer in critical human and financial resources away from implementing the Draft Strategic Plan announced in 2007, and toward implementation of the August 2010 “Public Health Emergency Medical Countermeasures Enterprise Review.” Although the Medical Countermeasures Review provides broad suggestions, it does not provide the necessary transparency to industry regarding what products are required, in what quantities, and paid for with what budgets, all of which had been outlined in the 2007 Draft Strategic Plan. This information is absolutely critical in order for industry to devote its scarce resources to the public health preparedness sector.

This is not to say that all of the recommendations of the MCM review are flawed or that reviews are unwarranted. However, this constant shift in priorities and funding, along with delays, has presented considerable uncertainty that has directly impacted the ability of companies to participate in medical countermeasure initiatives. This lack of transparency is an enormous barrier to long term private sector interest in working the U.S. government on medical countermeasures.

Moreover, the continued delays in both issuing requests for proposal and awarding contracts have placed tremendous pressure upon industry to justify its continued participation in the U.S. funded public health efforts. As you can imagine, in these financial times, when the management of a biotech, no matter the size, cannot tell its investors when an opportunity is coming and how much the opportunity will be potentially worth to the company, the resources dedicated in pursuit of that effort will be cut, plain and simple. The solution to this problem is not to make the function into a government-run entity, as some have suggested, but rather to

adjust the government's performance to maximize private sector participation as envisioned by Project BioShield.

To exemplify this point, consider that there are currently four FDA approved products that have the immediate potential to benefit victims of a nuclear incident - regardless of whether it was caused by nature, as in what has happened in Japan, or detonation of a improvised nuclear device, an event the Co-Chairs of the 9/11 Commission described as "certain" to occur in their lifetime. Three of these products are made by the two of the largest of biotechnology companies in the world and have not only been on the market for over 10 years, but have been used in nuclear accidents in the past. The BARDA leadership is well aware of these products and is eager to see procurement of these products move forward. Yet after over two years of discussions, no Requests for Proposal have been issued to allow the government to acquire these products, even though the funding is currently available in the Special Reserve Fund under Project BioShield to do so.

I am aware that at least two of these companies are under extreme pressure from their management to justify any continued efforts in pursuing these projects due to these delays. One of those companies feeling this pressure is a small, yet well funded, biotech, whose investors view efforts to try to assist BARDA as an unwarranted distraction, even though this company has an FDA approved product that would have an immediate benefit to victims of a terrorist attack, as well as a natural disaster. Small biotechs are exactly the innovative engines the Government needs to address these public health problems. If these companies ultimately have to walk away due to these unwarranted delays, there is no question it will cost lives in the future.

The solution here is simple. There must be a clear statement of priorities, including allocation of resources and funding, with a realistic and achievable schedule for implementation that will actually be followed without delay. This does not require legislative change or even future appropriations. But it is absolutely critical in order for industry's participation in public health preparedness efforts to continue.

Turning to my second observation, there are several recent examples where a public health emergency has presented a situation that allows public officials to not only assess the ability of the U.S. to respond in a "live fire" exercise, but also to retrospectively, and proactively, examine what could be done better or could be learned from the event. I'm concerned that several of these situations have passed without proactive action to learn from the event. Let me offer three, specific examples to make this point.

The Subcommittee is well aware of the growing challenges facing Japan as well as the flurry of discussions these incidents have sparked regarding the state of U.S. preparedness for all three aspects – the earthquake, the tsunami, and the nuclear emergency - of the disaster. There are medical countermeasures currently available as well as products under development in the U.S., many of which are funded by BARDA and DOD, which could play an important role in one or more the elements of the response in Japan.

It is completely understandable that the U.S. cannot and should not act without being requested to do so by the Japanese government. It is equally understandable that BARDA cannot and should not be placed in the position of supporting the use of a non-FDA approved product outside of the authority provided by Project BioShield. However, it seems that it would be appropriate for BARDA to proactively reach out to its industry partners to 1) determine what

products, if any, are currently available should they be requested by Japan and in what quantities and location; and 2) should these products be requested for use in Japan, what type of protocols, including Phase 4 and Emergency Use protocols, need to be in place to ensure the products are used as safely and effectively as possible. Having this information in hand today is key to being able to respond immediately if a request for assistance is received from Japan (or any other country facing a nuclear incident), rather than having to delay the response while this information is collected in a reactionary fashion. None of these actions require a request by Japan from assistance, nor do they require legislative action or additional funding. Yet, based upon discussions with several of the relevant companies, this proactive outreach has not occurred.

In a similar vein, the Subcommittee may be aware that there have been several recent incidents of anthrax infection in heroin users in Scotland. The U.K. public health officials have faced unique challenges with these patients and have gained considerable insights into how different therapeutics have contributed to and failed to contribute to the survival of these patients. I personally met with the lead U.K officials handling this response in September of last year and, as you would imagine, they had a wealth of unique and valuable information regarding the course of the disease in these patients. I understand this information has been shared by the U.K. with their U.S. counterparts. But yet again, based upon, my discussions with industry and the U.K officials, there has yet to be a proactive effort by U.S officials to share the information and data gleaned from these incidents with the companies developing anthrax treatments, nor has it been shared with researchers who are working to understand disease pathogenesis.

Finally, in March 2009, there was a widely reported incident in San Diego where a young Marine developed Progressive Vaccinia, a virus that closely resembles smallpox, after

having received a smallpox vaccination. There was a tremendous response by military doctors, the Centers for Disease Control and Prevention, and the FDA to respond to this incident. There were multiple products used to treat this patient, including products currently in the Strategic National Stockpile as well as experimental products in late stage development for use in a smallpox incident. The lessons learned from this case are extremely valuable for understanding what a mass casualty event involving smallpox would look like, and for determining effective deployment of therapeutics. But yet again, based upon my understanding, there has been no affirmative outreach by BARDA or DOD to debrief the industry responders to understand what they learned from this incident. To the opposite, when BARDA was asked during the course of an active procurement by a prospective offeror to affirmatively consider the experience of the human use of these products in evaluating which products were most appropriate for stockpile, the request was declined.

These examples demonstrate a frustrating pattern where opportunities to learn are being lost and relevant information is not being even accumulated, much less considered. At the same time, companies that are being asked to propose to various procurement opportunities must develop a “Target Product Profile,” not only as part of their proposal, but more importantly, to guide the interactions with FDA. However, it is impossible to develop a TPP in the absence of an accurate understanding for how the product will be used in a public health emergency. This understanding can only be gained through a meaningful dialogue between industry and government – incidents such as those I’ve outlined present a very unique situation for such a dialogue that is being utterly missed.

My strong belief is this failure to be proactive is not a result of inaction or lack of forethought by the leadership at BARDA. Rather, the likely cause is an unnecessary and non-

productive interference with the ability of the BARDA leadership and program managers to communicate with industry by the perceived restrictions of the Federal Acquisition Regulations (FAR). I emphasize the word “perceived” given that based on the clear language of the FAR and my over sixteen years of experience in government contracts law (both inside and outside the government) there is absolutely nothing that prevents such interactions from taking place. To the contrary, as was recently made clear in a memorandum issued by Dan Gordon, President Obama’s head of the Office of Federal Procurement Policy, transparent interactions with industry are an essential part of the procurement system and should not be inappropriately constrained by Agency contracting officers. Despite this clear guidance from the top procurement officials in the Administration, it has been my experience that communications from the BARDA leadership and program managers has been unnecessarily constrained by the contracting officials to the significant detriment of BARDA’s mission.

In the past, the procurement function and the contracting officers themselves were part of BARDA, and thus, the BARDA Director had greater influence to ensure both transparent communication as well as proper allocation of priorities by the contracting officers supporting the procurement process for medical countermeasures. Just over a year ago, this function was moved outside of the direct supervision of the BARDA Director, as was the requirement setting process. Since this has occurred, there has been a marked decline in the speed and efficiency of the contracting process. Reverting back to the prior organization, where the BARDA Director has responsibility and accountability for the contracting officers and requirements process supporting BARDA, would be a welcome change that would not require any change in legislation or additional costs to implement. Further, increased Congressional oversight to

encourage greater proactive response from the Public Health Enterprise, as a whole, would most certainly be a benefit.

The final observation I'd like to discuss today is the need for there to be greater focus on the sustainability of the overall Public Health Enterprise to ensure the investments made by BARDA are maximized. Reauthorization of Project BioShield and the replenishment of the soon-to-be exhausted Special Reserve Fund is a key component of sustainability. I strongly encourage Congress to do both in conjunction with the reauthorization of PAHPA. That said, even without any legislative action or additional funding, it is incumbent upon the Public Health Enterprise to make the best use possible of the remaining balance of BioShield funding and other resources to ensure sustainability.

The first order of business must be to ensure that the products currently in the SNS are maintained at their current level. For products such as the licensed anthrax and smallpox vaccines, that means ensuring the CDC has both the funding and processes it needs to ensure the levels of non-expired vaccine in the stockpile, at a very minimum, are maintained. However, it should be a top priority that we stockpile levels of countermeasures to match the Material Threat Assessments conducted by the Department of Homeland Security in order to protect the civilian population, our first responders, and our military men and women should an event occur. For example, we currently fall far short of having adequate stockpiles of licensed anthrax vaccine to meet the stated 75 million dose requirement set by the Material Threat Assessment. Addressing this should be a priority.

For products that have yet to achieve FDA approval, including anthrax therapeutics and next-generation smallpox vaccines being procured under Project BioShield, that also means

BARDA must exercise the options in those contracts to retain the supply of unexpired products at the levels currently in the stockpile, as well as to ensure that the substantial investment BARDA has made in the manufacturing capacity to support those products is not lost. Given that BARDA has recently undertaken an effort to create multiple “Centers of Innovation for Advanced Development and Manufacturing” to supplement the Nation’s manufacturing capacity, an effort that is expected to take decades and cost billions to achieve, it seems the first, near term step in maintaining a viable manufacturing capacity for medical countermeasures must begin with ensuring the investments made in the current capacity are not lost.

Next, the Medical Countermeasures Review correctly placed significant importance upon the need to procure broad spectrum, dual use products – that is, products that have both a CBRN and commercial use. These products will be, by definition, more likely to achieve FDA approval given that the human data derived to support the commercial indication will supplement the animal data needed for approval under the Animal Efficacy Rule for the CBRN indication. Once approved by FDA for a commercial indication, the cost to the government to sustain these products for CBRN use is also far lower than the need to re-procure and stockpile products that are only usable in the event of a public health emergency. Although the benefits of dual-use technologies are clear, and are articulated in the 2010 Medical Countermeasures Review, it appears that products that lack this dual-use potential are still being favored for procurement under Project BioShield. This lack of consistency with the clear mandate of Medical Countermeasures Review is puzzling to say the least.

Given the investment in creating and staffing the organization, BARDA should also have a clear role in the response to non-biodefense threats to public health such as the rise of multi-drug resistant pathogens – the “super bugs” that are killing far more people every year than the

losses we suffered on 9/11. Emerging tropical diseases like dengue and global health diseases such as tuberculosis are also impacting the United States, with a growing number of cases of dengue and TB in Florida, Hawaii and elsewhere. BARDA should play a significant role addressing these diseases. The investment in the infrastructure to create and support BARDA, as well as the obvious benefits and synergies of expanding the mission to include emerging infectious disease, make clear this is a worthy focus for BARDA. This is the one area where I believe Congress should affirmatively act to modify the PAHPA legislation to explicitly give BARDA the mandate to address drug resistance – both bacterial and viral – as well as emerging infectious disease as a whole. This may require additional appropriations to support this expanded mission, but it is an area that needs to be addressed and BARDA is ideally suited to take on this mission.

In closing, I would like to return to both the 2009 influenza pandemic as well as the events in Japan.

On the morning of September 11, 2001, a trusted advisor to the Secretary of HHS had a meeting scheduled with the Secretary to raise the issue of the emergence of the H5N1 virus in Asia and how the U.S. should prepare for an influenza pandemic like the one that devastated the world in 1918 as described in John Barry's book "The Great Influenza." That meeting never occurred that day for obvious reasons, however, it was eventually rescheduled. HHS went on to make critical investments to secure the egg supply for flu vaccines, to bolster the U.S. vaccine base, stockpile millions of doses of flu antivirals, as well as diagnostics, and to support the passage of legislation to address liability issues that up-to-then had restrained our ability to prepare. That trusted advisor became the first Assistant Secretary for Preparedness, where, as the precursor to what is now BARDA, he made critical decisions regarding influenza vaccines

and therapeutics, anthrax vaccine and therapeutics, smallpox vaccines, and radiation countermeasures. These decisions were implemented by a skeleton staff made up mostly of detailees from other parts of HHS and retired public health leaders who offered their time in order to help protect the nation. The procurements were managed by a single contracting officer at the CDC, for which this was an extra duty. About half of those decisions, in retrospect, ultimately did not result in outcomes that immediately protected the homeland. However, about half of them did. The Assistant Secretary withstood enormous criticism for the decisions that did not appear to be immediately beneficial, and got little credit for the decisions that proved right, including those critical decisions that helped prepare the Nation for the 2009 pandemic. Mr. Chairman, as the baseball teams that have Spring Training in your district are aware, a .500 batting average is something to be proud of. The bottom line is decisions were made then that clearly protected the United States. Yet, today, decision making is ground to a halt by concerns about the perception that could result from a failure and overly bureaucratic procedures while the security of our homeland suffers.

If we look toward to Japan, the lack of proactive decisions to inventory what drugs are currently available to respond to a nuclear emergency, of not ensuring that well-conceived protocols are written in advance to ensure their appropriate deployment if these products are ever used, and of failing to hear the wakeup call the events of the last month signal for the need to prepare in America could prove devastating. The decisions made to today – or better put, the decisions that are not being made today – will almost certainly result in leaving our Homeland vulnerable. I thank you Mr. Chairman and this Committee for doing everything you can to ensure that our Homeland remains secure.