Statement PhRMA

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FOR THE

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BEFORE THE

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The Pharmaceutical Research and Manufacturers of America (PhRMA) appreciates the opportunity to share with this Committee the views of the research-based pharmaceutical industry on countering the bioterrorism threat and on the Project Bioshield initiative.

PhRMA represents the country's leading research-based pharmaceutical and biotechnology companies, which invested an estimated $32 billion in 2002 in developing new medicines to help and heal patients. PhRMA member companies join others who are convinced that biological weapons present a serious and increasing danger to people around the world. The pharmaceutical industry is dedicated to the development of innovative therapies and vaccines to counter unmet medical needs. Because a substantial proportion of the unmet medical need in the United States and worldwide is both directly and indirectly related to

infectious diseases, we understand the seriousness of the threat of biological agents if used as weapons of war.

The complexity of the problem of biological weapons is amply demonstrated by science's continuing difficulty in dealing with infectious agents as the cause of natural disease. The threat represented by infectious diseases – such as HIV, malaria, and tuberculosis – is real and all too well demonstrated by the deaths of over 5 million people annually from these three diseases alone. All together, infectious diseases claim more than 100,000 American lives each year and cost more than $30 billion annually in direct treatment expenses alone. At last count, PhRMA member companies were developing 256 new medicines to treat or prevent infectious diseases – medicines which include brand new classes of antibiotics, new vaccines (including edible vaccines), antifungals, antivirals, and immune enhancers.

Particularly in light of continuing difficulties in infectious agent research, the potential use of these agents in intentional concentrated exposures of targeted populations raises grave concerns. Reports from the National Academy of Sciences, the NIH Blue Ribbon Panel for Biodefense Research, and the US Defense Science Board make clear that a large number of countermeasures to biothreats must be developed. Indeed, existing medicines are not sufficient to combat the biological weapons already developed. Needed countermeasures will include vaccines, therapeutics, and diagnostics.

The basic science research required for countermeasure development has already been stimulated by funds appropriated to various federal agencies

including the Department of Health and Human Services and the Department of Defense. It is widely recognized, however, that a cooperative and collaborative research and development effort, which engages industry, government, and academia, will be essential to the development of a complete arsenal of countermeasures against bioterrorism agents.

PhRMA and its member companies are already working closely with federal agencies and academia to move forward with this research. For example, PhRMA is working with CDC, DoD, NIH, FDA, and academia to support in vitro studies of five pathogens – B. anthracis (anthrax), Y. pestis (plague), Brucella spp. (brucellosis), F. tularensis (tularemia), and Burkholderia spp. (Glanders) – for testing of existing antibiotics. Several companies are working with the National Institute of Allergy and Infectious Diseases (NIAID), the Department of Defense, and the FDA to test existing antibiotics against plague, and PhRMA will cosponsor a workshop with interested parties to determine how best to expand labeling of other existing antibiotics that may be effective against the top biothreat agents. PhRMA committees continue to work with FDA to clarify and improve existing regulations that pertain to biothreat countermeasure research, such as the "Spore Formers Rule," 21 C.F.R. Part 610, which imposes requirements on use of facilities or equipment that have been used with spore forming organisms, and the "Animal Rule," 21 C.F.R. § 314.610, which allows efficacy testing in animals where testing in humans would be impossible or unethical. We have prepared educational materials for the public on anthrax, smallpox, and vaccinia, and we are working on materials addressing tularemia

and plague. Dr. Gail Cassell, PhRMA's Chief Scientific Officer for Emergency Preparedness and Vice President, Scientific Affairs at Eli Lilly & Co., sits on Secretary Thompson's Advisory Council on Public Health Preparedness. A Biosurveillance workgroup involving PhRMA, other private sector companies (TIGR, IBM, and Roche Diagnostics), federal agencies (CDC, DoD, and NIH), and the World Health Organization is working to establish a global infectious disease electronic surveillance network.

Project Bioshield, announced by President Bush in his 2003 State of the Union address, is an important step forward in the effort to ensure the development of modern, effective countermeasures and to ensure that these products become available in a timely and efficient manner. PhRMA generally supports the three main components of the President's proposal: first, the creation of a permanent indefinite funding authority to spur the development of medicines and vaccines by the private sector; second, new authority for NIH to speed promising R&D through streamlined hiring and procurement mechanisms and increased flexibility to award contracts and grants; and third, new FDA emergency use authorization for promising treatments still under development.

Any legislation to implement the President's initiative must – if it is to be successful – take into account the significant scientific, legal, and economic impediments to the research and development of biodefense products.

Research and development into new medicines is itself a lengthy, risky, and expensive endeavor. Bringing a drug from concept to market takes 10 to 15 years. The average cost to develop a new drug has grown from $138 million in

1975 to $802 million in 2000. The risks involved in the new drug development and approval process are substantial. Of every 5000 compounds screened, only 250 enter preclinical testing, and of every 250 drugs that enter preclinical testing, only one is approved by FDA. Only 3 of 10 marketed drugs produce revenues that match or exceed average R&D costs.

Moreover, research into biothreat countermeasures involves many challenges above and beyond those encountered in non-biodefense R&D. For example, biodefense R&D requires working with dangerous pathogens in highly specialized facilities, and developing countermeasures without a full picture of the risk of disease (because we cannot see into the mind of the terrorist) or the benefit of the treatment (because there are often no patients with the disease, which prevents clinical testing for efficacy).

The decision to divert resources from the research and development of medicines for serious illnesses like heart disease also can be financially risky, especially when a countermeasure may never be purchased or used, and especially for companies with few products in the pipeline. (Diverting resources from research and development of these other medicines will also affect the future availability of treatments and cures for patients with other serious health conditions — especially since less than ten percent of all drugs that enter testing ever demonstrate sufficient safety and acceptable efficacy.)

Finally, manufacturers that develop countermeasures may be exposed to devastating product-liability suits. Some of these would arise out of adverse events that are unavoidable given the nature of the products, and some could

arise simply because the products were made available without the usual battery of clinical trials required for FDA-approved products. Private insurance could be unavailable or prohibitively expensive for such products.

In light of the special obstacles to research and development in the bioterrorism context, PhRMA has developed recommendations for any legislation that would implement Project Bioshield.

First, PhRMA believes that meaningful liability protection is an essential component of any legislation to encourage the development of bioterrorism countermeasures. Provisions in current law – namely the SAFETY Act, 6 U.S.C. §§ 441-444, and the indemnification available under Public Law 85-804 – are associated with too many uncertainties, limitations, and conditions to make them effective in this unique context. Accordingly, PhRMA supports liability protection modeled on either the Swine Flu legislation or section 304 of the Homeland Security Act.

Second, in order to engage the private sector most efficiently and effectively in this research, the procurement process must be more flexible and reliable, and it must more closely resemble the private market. We have a number of suggestions in this regard, the most significant of which follow: (1) the Secretary of Health and Human Services should be given flexible "other transactions authority" similar to that given to the Department of Defense, particularly the Defense Advanced Research Projects Agency, under 10 U.S.C. § 2371; (2) the legislation should provide that procurement contracts may recognize that pricing should take into account the actual cost of development

including costs incurred after contract execution; (3) it should expressly provide that the Secretary may enter into single contracts for both R&D and production; (4) it should permit the Secretary to purchase antibiotics and antiviral agents that have potential uses other than as countermeasures; (5) the Secretary should be authorized to include performance-based (milestone) payments in procurement contracts – rather than limited to repayable "advance payments" and payment conditioned on "substantial delivery"; (6) contracts should not be subject to termination at the convenience of the government or for non-delivery within a fixed statutory period; and (7) contracts should not be limited to countermeasures that can be developed within five years. We have been discussing these provisions with members of the Administration and members of Congress, and we look forward to continuing these discussions so as to work toward the inclusion of these provisions in the legislation.

Finally, although the overall model of bioterrorism countermeasure research and development should build on competition among private companies, the need for urgent development of medicines may require the sharing of information and cooperation among companies, which can raise antitrust concerns. PhRMA therefore believes that it would be appropriate to provide a narrowly tailored antitrust exemption to facilitate certain meetings and activities, under careful governmental safeguards.

A strong commitment from all parties will be necessary in the months and years to come, as our nation seeks to protect itself against the terrible threats of

biowarfare and bioterrorism. America's pharmaceutical companies look forward to doing our part.

We thank you for your time and look forward to answering your questions.