PhRMA

STATEMENT OF
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BEFORE THE
PRESEIDENTIAL COMMISSION FOR THE STUDY OF BIOETHICAL ISSUES

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Distinguished members of the Commission and staff, my name is Jeffrey Francer, Assistant General Counsel of the Pharmaceutical Research and Manufacturers of America ("PhRMA"). PhRMA is pleased to provide additional testimony today to supplement our written comments submitted to the docket in May of this year.

PhRMA represents the country's leading pharmaceutical research and biotechnology companies, which are devoted to inventing medicines that allow patients to live longer, healthier, and more productive lives. PhRMA companies are leading the way in the search for new cures. PhRMA members alone invested an estimated $49.4 billion in 2010 toward discovering and developing new medicines. Industry-wide research and investment reached a record $67.4 billion in 2010.

PhRMA and its member companies are firmly committed to conducting high quality, scientific, and ethical clinical research in a manner that respects and protects the rights, dignity, safety and welfare of all study participants in full compliance with applicable laws and regulations wherever in the world clinical trials are performed. PhRMA and its member companies believe that a meaningful research objective, a scientifically valid study design, adherence to fundamental ethical principles, respect for local norms and culture, compliance with applicable legal and regulatory requirements, and high quality protocol execution will facilitate clinical research that is valid, reliable and ethically acceptable globally.

In my testimony today, I will focus on three areas:

(1) PhRMA's support of educating clinical investigators on the values and rationale that support human subject protection laws and regulations;

(2) Support for harmonizing human subject protection regulations; and

(3) Support for government-funded coverage of injuries that result from a government-funded clinical trial and providing by way of example, the typical practice of reimbursement for injuries caused by investigational drugs in trials sponsored by PhRMA's member companies.

I. PhRMA's Support of Educating Clinical Investigators on the Values and Rationale that Support Human Subject Protection Laws and Regulations

PhRMA commends the Commission for initiating a thorough review of the current rules and standards for protecting human subjects in scientific studies in the wake of recent revelations regarding government support of research in Guatemala from 1946 to 1948 involving the intentional infection of vulnerable populations with sexually transmitted diseases. As President Obama observed, these revelations offer a "sobering reminder of past abuses."

While the United States government's past support of the unethical research in Guatemala in the 1940's is shameful, it does not reflect the current regulatory regime or current industry or government practices. The existing standards for human subject protection are significantly different than those in force during the time the Guatemala research was conducted and are specifically designed to prevent similar abuses from recurring in the future. PhRMA agrees with Secretary of State Hillary Clinton and Secretary of Health and Human Services Kathleen Sebelius that "the regulations that govern U.S.-funded human medical research [today] prohibit these kinds of appalling violations."[1]

In order to help ensure that future government studies do not repeat abuses such as those that occurred in the Guatemala studies, PhRMA supports enhanced educational efforts aimed at future clinical investigators around the values behind today's complex rules and regulations governing clinical research.

In 2002, PhRMA adopted its *Principles on Conduct of Clinical Trials and Communication of Clinical Trial Results* ("PhRMA Clinical Trial Principles"). These Principles, which are based on standards established by the Declaration of Helsinki and the International Conference on Harmonization's ("ICH") Guideline for Good Clinical Practice, reinforce the biopharmaceutical industry's "commitment to the safety of research participants" and "to sponsoring clinical research that fully complies with all legal and regulatory requirements."[2] The PhRMA Principles have been revised twice since their adoption in 2002.

PhRMA believes that government and private sponsors could use materials such as the PhRMA Clinical Trial Principles, and perhaps other co-developed materials, as resources for enhanced training of clinical investigators. PhRMA would be happy to work with the Department of Health and Human Services ("HHS") in designing and implementing such a program.

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[1] *See* Joint Statement by Secretaries Clinton and Sebelius on a 1946-1948 Study (Oct. 1, 2010) *available at* http://www.state.gov/secretary/rm/2010/10/148464.htm.

[2] Pharmaceutical Research and Manufacturers of America, *Principles on Conduct of Clinical Trials and Communication of Clinical Trial Results*, at 2-3 (2009) (hereinafter "PhRMA Principles")

II. PhRMA's Support For Harmonizing Human Subject Protection Regulations

The development of innovative therapies to treat disease and improve the quality of life is a long and complex process that is fraught with risk. It takes on average 10 to 15 years and more than $1.2 billion to bring a single new medicine to the market.[3] Moreover, only 1 in 5,000 to 10,000 compounds identified in the laboratory makes it through the development process and obtains approval from the Food and Drug Administration ("FDA").[4] Inefficiencies in the development process – including non-harmonized regulatory requirements – can increase the time it takes to develop new medicines as well as potentially increase the number of research subjects required in a global development program.

The majority of the clinical research conducted by biopharmaceutical companies in the United States is governed by regulations adopted by the FDA. The FDA has promulgated comprehensive regulations governing the clinical trial process, including the requirements for submitting and maintaining an effective IND,[5] the requirement to obtain approval from an independent Institutional Review Board ("IRB"),[6] and the requirement to obtain informed consent from study subjects.[7] Together, these regulations often are referred to as the Good Clinical Practice, or GCP, requirements.

In addition to FDA's regulatory regime, HHS and fourteen other Federal departments and agencies have adopted comprehensive regulations governing clinical trials that are conducted or supported by such departments or agencies.[8] These regulations, collectively known as "the Common Rule," likewise adopt the GCP requirements of informed consent; IRB review, approval and monitoring; and government review and oversight of research activities. As discussed in PhRMA's earlier testimony, these GCP requirements are comprehensive and serve

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[3] DiMasi, JA, and Grabowski, HG. The cost of biopharmaceutical R&D: Is biotech different? Managerial and Decision Economics; 2007(28): 469-479.

[4] Drug Discovery and Development: Understanding the R&D Process. www.innovation.org; Congressional Budget Office (CBO). Research and Development in the Pharmaceutical Industry, Washington, DC: CBO, October 2006.

[5] *See* 21 C.F.R. Part 312.

[6] *See* 21 C.F.R. Part 56.

[7] *See* 21 C.F.R. Part 50.

[8] *See* 45 C.F.R. Part 46. The Common Rule has been adopted by the Department of Agriculture (7 C.F.R. Part 1c); the Department of Energy (10 C.F.R. Part 745); the National Aeronautics and Space Administration (14 C.F.R. Part 1230); the Department of Commerce (15 C.F.R. Part 27); the Consumer Product Safety Commission (16 C.F.R. Part 1028); the Agency for International Development (22 C.F.R. Part 225); the Department of Housing and Urban Development (24 C.F.R. Part 60); the Department of Justice (28 C.F.R. Part 46); the Department of Defense (32 C.F.R. Part 219); the Department of Education (34 C.F.R. Part 97); the Department of Veterans Affairs (38 C.F.R. Part 16); the Environmental Protection Agency (40 C.F.R. Part 26); the National Science Foundation (45 C.F.R. Part 690); and the Department of Transportation (49 C.F.R. Part 11). The Central Intelligence Agency also must comply with the Common Rule pursuant to Executive Order 12333, and the Department of Homeland Security has chosen to apply the Common Rule to its human research activities.

to protect the rights, health and safety of research participants in clinical trials conducted or supported by the Federal government. Moreover, these requirements apply not just to domestic clinical investigations but also to studies conducted in foreign countries.

PhRMA supports harmonization of clinical development regulations, including requirements for human subject protection. Multiple development programs to satisfy regulatory requirements in different regions of the world are not practical or sustainable, and significantly, requirements for multiple development programs may serve as a substantial disincentive to the development of the most urgently needed medicines worldwide. In addition, given the ethical nature of human subject protection, different regulations across the Federal government and around the world appear to make little sense. We note that HHS has recently issued an advance notice of proposed rulemaking regarding potential changes to the Common Rule. We urge HHS to ensure that the Common Rule is fully harmonized with the FDA's IND and IRB regulations and other global standards. Different sets of requirements in this area are unnecessary and would increase the difficulty of training of clinical investigators on human subject protection standards.

III. Compensation for Clinical Trial Injuries

Finally, the Commission staff has asked PhRMA to provide testimony on the typical practice of pharmaceutical company sponsors providing compensation for injuries caused as a result of investigational drugs used in clinical trials. To that end, PhRMA performed an informal survey of our members in order to inform the Commission.

PhRMA's members typically commit at the outset of a clinical trial to pay the cost of medical care provided to treat injuries incurred by research participants that are caused by investigational drugs during a clinical trial. If companies commit to provide such compensation, they may commit to pay either the cost of all medical care required to treat injuries caused by investigational agents or the cost of medical care not reimbursable by a third party payer.

PhRMA would support government-funded reimbursement for injuries that result from government-funded clinical trials, and we believe that such a practice would be ethical and consistent with typical practice among our member companies.

Conclusion

In conclusion, PhRMA believes that the Guatemala study conducted by the U.S. government in 1946-1948 marks a dark chapter in human research; yet robust and comprehensive Federal and international regulatory and ethical standards and protections have been enacted and implemented during the last sixty years.

We applaud the Commission and its staff for their thorough examination of this incident and your consideration of relevant policy proposals to guide future U.S. government-sponsored clinical trials.

PhRMA appreciates your consideration of our testimony, and I would be happy to answer your questions.