



# Statement of the U.S. Chamber of Commerce

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**ON:** PROPOSED RECOMMENDATIONS FOR THE  
REAUTHORIZATION OF THE PRESCRIPTION DRUG  
USER FEE ACT FOR FISCAL YEARS 2008 TO 2012  
("PDUFA IV")

**TO:** THE FOOD AND DRUG ADMINISTRATION

**BY:** ANTHONY C. WISNIEWSKI

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The Chamber's mission is to advance human progress through an economic,  
political and social system based on individual freedom,  
incentive, initiative, opportunity and responsibility.

The U.S. Chamber of Commerce is the world's largest business federation, representing more than three million businesses and organizations of every size, sector, and region.

More than 96 percent of the Chamber's members are small businesses with 100 or fewer employees, 70 percent of which have 10 or fewer employees. Yet, virtually all of the nation's largest companies are also active members. We are particularly cognizant of the problems of smaller businesses, as well as issues facing the business community at large.

Besides representing a cross-section of the American business community in terms of number of employees, the Chamber represents a wide management spectrum by type of business and location. Each major classification of American business -- manufacturing, retailing, services, construction, wholesaling, and finance -- is represented. Also, the Chamber has substantial membership in all 50 states.

The Chamber's international reach is substantial as well. It believes that global interdependence provides an opportunity, not a threat. In addition to the U.S. Chamber of Commerce's 105 American Chambers of Commerce abroad, an increasing number of members are engaged in the export and import of both goods and services and have ongoing investment activities. The Chamber favors strengthened international competitiveness and opposes artificial U.S. and foreign barriers to international business.

Positions on national issues are developed by a cross-section of Chamber members serving on committees, subcommittees, and task forces. More than 1,000 business people participate in this process.

**Statement of Anthony C. Wisniewski**  
**Executive Director of Health Care Policy**  
**for the**  
**United States Chamber of Commerce**  
**Before the**  
**Food and Drug Administration**  
**February 16, 2006**

Members and representatives of the Food and Drug Administration, I am pleased and honored to be here today. Thank you for your kind invitation.

By way of introduction, I am a life sciences attorney serving as the head of health care policy for the United States Chamber of Commerce (“Chamber”), where my responsibilities include managing the Chamber’s diverse health policy portfolio and bearing primary responsibility for health policy issues pending before the U.S. Congress and the federal agencies.<sup>1</sup> Prior to joining the Chamber, I served as the head of public policy for biotechnology company MedImmune, and prior to that worked in the life sciences industry on behalf of an industry trade association as well as focusing on corporate transactions and government affairs while in private legal practice. I also currently serve as a gubernatorial appointee to the Board of Directors of the Maryland Technology Development Corporation, a public/private entity that is a national leader in providing seed stage capital to emerging biotechnology companies.

**Synopsis of the Chamber’s Position on the PDUFA IV Proposal**

The U.S. Chamber of Commerce’s membership realizes the exigency of controlling the cost of accessible, quality employer-sponsored health care, while adhering to the Chamber’s principles of: promoting entrepreneurship; safeguarding the fruits of its

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<sup>1</sup> The Chamber is also considering launching a new Life Sciences Study Group dedicated to educating, developing policy and engaging in advocacy on legislative and regulatory issues that concern the pharmaceutical, biotechnology, medical device and life sciences venture capital industries.

success with reliable intellectual property protections; and encouraging future discovery and innovation through a free-market business environment.

Accordingly, the Chamber applauds the historic successes of PDUFA. PDUFA's programs have dramatically improved the drug approval process, remedying the significant "drug lag" of the 1990's. However, these accomplishments in the pre-market product review process have also made PDUFA an attractive vehicle for increasing user fees to fund other programs such as post-market safety surveillance and most recently direct-to-consumer advertising. Generally speaking, we believe that the PDUFA IV proposal will ensure that FDA maintains the necessary tools for conducting timely yet thorough reviews for new drugs.

Given some incidents of advertised, popular products that have been recalled in the past five years, ensuring drug safety has become paramount. Improving post-market drug safety and its funding must be a fundamental priority.

However, there must be a balance between safety and access. Without this balance, consumer access to critical new treatments will be delayed. This "chicken-and-egg" issue begs the question: How stringent can the approval process afford to be before it hinders entrepreneurial discovery and the innovation of new medicines? Onerous regulations could eventually frustrate a biotech's ability to attract venture capital funding. Likewise, established innovator companies may not be able to prevent a higher cost of bringing and maintaining drugs on the market without passing added cost unto the consumer.

The Chamber believes that the FDA's and the PDUFA stakeholders' assertion is correct: providing guidance to the industry on clinical trial design will improve the development of new drugs. Likewise, the Chamber also agrees that focusing efforts on useful trials and decreasing less useful experimentation will reduce R&D times and total costs of bringing a new drug to market, all of which should lower drug prices for the consumer.

## **Critical Path: A proactive idea for new product discovery**

The Chamber also supports collecting user fees to promote scientific research collaborations designed to clarify regulatory pathways for new technologies and potential new biomarkers for drug safety and effectiveness. However, in this area of the PDUFA IV proposal, we would like to provide added encouragement that Critical Path projects receive adequate user fees and perhaps also appropriations funding to help accelerate new drug development.

Critical Path programs are personalized medicine and pre-market-focused vehicles designed to address drug safety. Yet these programs potentially offset the cost of compliance with the inclusion of additional post-market regulatory requirements. Utilizing PDUFA's user fees to more clearly fund these projects can decrease the cost of clinical trials, expedite development of new medicines, reduce the number of adverse events and related litigation costs, and provide greater access to consumers at an ostensibly lower cost. While we certainly do not expect Critical Path projects in any way to displace post-market safety priorities, we do believe that more appropriately addressing Critical Path funding would provide a greater balance between access and safety concerns.

In October 2006, the Congressional Budget Office ("CBO") released its report, "R&D in the Pharmaceutical Industry." This report finds that the drug industry spends more on R&D, relative to its sales revenue, than almost any other industry in the United States. Based on the results of several independent studies, the report asserts that the industry's real (inflation-adjusted) spending on drug R&D has increased between threefold and six fold over the past 25 years. However, the CBO report goes on to say that in spite of increases in R&D spending, "...there has been little change in the number of innovative new drugs approved for use each year..."<sup>2</sup>

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<sup>2</sup> Congressional Budget Office, Pub. No. 2589, "R&D in the Pharmaceutical Industry," p.7 (October 2006).

Greater research challenges, more technological complexities in drug development and greater specificity in disease targets all contribute to increased R&D expenditures, as well as higher clinical trial failure rates.<sup>3</sup> The FDA reports that the proportion of all eventually approved drugs entering Phase I clinical trials has dropped from a historical average of about 14 percent to 8 percent.<sup>4</sup> These failures contribute to increasing R&D costs per new product, especially when those failures occur further down the clinical trial process.

As a result, under the current clinical trial system, many products are failing to reach the market because they cannot be proved safe or effective, or because they could not be manufactured at a consistently high quality. The Chamber agrees with FDA that a large part of the blame falls upon the scientific tools needed to develop new medicines that are trailing the rapid advances in new medicine discovery.<sup>5</sup> Specifically, the existing preclinical and clinical testing of drugs has proven time-consuming, inefficient and very expensive, which is estimated to cost the industry between \$0.8-\$1.7 billion and 12-15 years of R&D to bring a product to market. This inefficient pre-market process delays consumer access to new medicines and drives up market prices.<sup>6</sup>

FDA's development of the Critical Path Initiative in March 2004 focuses on incorporating modernized "process-oriented" scientific tools. These tools include biomarkers, innovative clinical trial designs, simulation models of physiology and disease processes, and manufacturing assessment methods.<sup>7 8</sup> Such tools have the potential to evaluate safety and efficacy of new medicines expeditiously, with greater certainty, at lower costs and with better information.<sup>9</sup>

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<sup>3</sup> *Id.* at 2.

<sup>4</sup> Food and Drug Administration, "Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products," p.8 (March 2004).

<sup>5</sup> Rados, Carol, "The Critical Path: Accelerating the Development of Medical Products," *FDA Consumer Magazine*, p.1, (September-October 2004 issue).

<sup>6</sup> Critical Path Institute website, <http://www.c-path.org/>. Accessed 12/11/06.

<sup>7</sup> This includes promoting innovative clinical trials and statistical techniques that reduce the number of patients that receive placebo or to adaptively change the trial based on ongoing results.

<sup>8</sup> This includes quality assessment technologies that are used to analyze product quality (e.g. durability of devices, stability of drugs).

<sup>9</sup> Food and Drug Administration, "Critical Path Initiative Fact Sheet," FDA website, [http://www.fda.gov/oc/initiatives/critical\\_path/factsheet.html](http://www.fda.gov/oc/initiatives/critical_path/factsheet.html). Accessed 10/25/06.

Biomarkers are particularly interesting scientific tools that can be used to prevent, detect and treat a wide range of diseases, ranging from cancer to depression. Biomarker technology could save time and money in R&D, shorten clinical trial times while using fewer patients, and expedite final FDA marketing approval.<sup>10</sup> We are pleased to see that the “Biomarkers Consortium,” led by the National Institutes of Health Foundation (which awards the grants under the program) and FDA is collaborating with private industry groups to finance biomarker research.

The Critical Path Institute (“Institute”) is involved in similar “process-oriented” projects but does not accept funding from biopharmaceutical companies. Instead the Institute accomplishes its work by being a catalyst for coalitions of stakeholders to work together. We view the Institute’s “Fast Path” program as coinciding directly with the Chamber priority of lowering employer health care cost through, among other things, reducing R&D expenses. Fast Path partners the Institute with FDA, NIH, universities and other partners to research biomarkers, gene sequencing, and modified clinical trial design as ways to accelerate R&D processes. Particularly relevant Fast Path projects include:

- Cancer Biomarkers- Bringing together biopharmaceutical and diagnostic/medical device companies with government agencies to validate cancer-related biomarkers and to jointly develop predictive diagnostics and targeted therapies.
- Cardiovascular Genomics Project- Focusing upon congestive heart failure: the most common cause of hospitalization in the United States.<sup>11</sup> The Institute is collaborating with the FDA and the University of Utah to accelerate the impact of scientific innovations to treat CHF. Treatments include using DNA gene sequencing technology to predict what medicines will likely produce clinical improvements in patients with CHF and which patients who are at greatest risk of

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<sup>10</sup> This biomarker research correlates to another FDA priority on the medical device side: advancing new imaging techniques. Imaging can be used in conjunction with biomarkers to better treat cardiovascular disease, arthritis, neurocognitive disease, cancer and chronic obstructive pulmonary disease.

<sup>11</sup> Over five million Americans have CHF, 500,000 are newly diagnosed each year, and 250,000 die annually. CHF often occurs as a result of diabetic heart disease. Critical Path Institute website. <http://www.c-path.org/Programs/FastPath/tabid/60/Default.aspx>. Accessed 12/01/06.

adverse drug reactions. Tools that predict drug response in patients will significantly reduce the time frame and expense of R&D, and improve drug safety, lowering the cost of biopharmaceutical therapy for employers and consumers.<sup>12</sup>

Of course, there is already precedent for the successes of Fast Path projects in promoting drug safety and lowering the cost of health care. In the National Warfarin Study, the Institute is helping provide vital information to the FDA so they can develop drug labeling that will instruct physicians that patients with a certain genomic make-up should start with either a higher or lower dose of cardiovascular disease medication such as potentially life-saving blood thinners. Dosing adjustments would be based on an innovative clinical trial that would determine the value of using genetic testing to determine the best dose of Warfarin for each patient. These genomic-based dosing schedules will eliminate too high initial dosing (which causes bleeding) and too low dosing (which causes stroke). The AEI-Brookings Joint Center for Regulatory Studies concluded that this project will save lives and reduce health care spending by an estimated \$1.1 billion annually.<sup>13</sup> We are further supportive of the role the Institute plays in analyzing generic drugs such as Warfarin.<sup>14</sup>

These “Fast Path” projects may receive broad support as a means to reduce the time and expense of employing aging clinical trial and R&D processes that takes up to fifteen years to bring a product to marketing approval. Employing efficient tools will shorten product R&D times, reduce the number of costly failures, and potentially lower biopharmaceutical costs to the health care system. The Critical Path Institute provides scientific credibility and neutrality to the drug development process and applying user fees to fund the Institute’s programs will appeal to legislative overseers. Further, these Critical Path programs demonstrate FDA’s commitment to reducing biopharmaceutical R&D costs while promoting safety and consumer access to new medicines.

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<sup>12</sup> *Id.*

<sup>13</sup> AEI-Brookings Joint Center for Regulatory Affairs, Andrew McWilliam, Randall Lutter, Clark Nardinelli, “Health Care Savings from Personalized Medicine Using Genetic Testing: The Case of Warfarin.” Working Paper 06-23, p.3 (Nov 2006).

<sup>14</sup> Ratner, Mark, “Looking for solid ground along the Critical Path,” *Nature Biotechnology*, p.5, August 3, 2006. <http://www.nature.com/news/2006/060731/full/nbt0806-885.html>. Accessed 12/01/06.



We believe that directing user fee funding for Critical Path projects will add balance to the emerging trend of increased post-market safety surveillance and implementation of risk management plans. Over time, the projects' results may offset the increased manufacturer costs associated with additional safety and post-market obligations. Of course, cutting-edge scientific solutions to reducing pre-market R&D times for new products should increase consumer's access to more affordable drug therapies.

### **PDUFA for the Generic Drug Industry**

FDA should also support the adequate funding for its Office of Generics. As demonstrated through popular discount generic drug programs at large retail stores, generic drugs offer additional choices in drug therapies and enhance competition in the marketplace, ultimately making drugs more affordable to employers and consumers.

To address this concern, a "generic PDUFA" program should be further discussed. A public/private collaborative solution to funding the review and approval of generic drugs, complete with the deliverables and benchmarks of a PDUFA program, is the preferred method. Given that the medical device industry also pays user fees through their own Medical Device User Fee Modernization Act, it seems appropriate to perhaps consider a generic PDUFA program.

### **Conclusion**

Reauthorizing PDUFA is only one part of protecting innovators with promising new chemical compounds and molecular discoveries. It ensures a clean and timely review process for new products of both emerging and well-established innovators. Efficient regulatory review is the critical third component alongside the venture capitalists that fund new innovation and the entrepreneurial innovators that develop the

product. All three are necessary to ensure that new drug therapies are brought safely and efficiently to consumers and employers at the lowest possible cost.

While we applaud the terms of the PDUFA IV proposal we call upon FDA to give added consideration to ensuring that Critical Path projects receive adequate funding to accelerate new drug development. It is critical that consumers have optimal access to safe and lower cost innovative medicines. We also support further discussion of the creation of a new user fee program for the generic drug industry in order to accelerate the delivery of cost-effective alternative drug therapies to consumers and employers.