

July 30, 2004

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Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane
Room 1061
Rockville, MD 20852

Submitter: Greg Simon
Organization: FasterCures / The Center for Accelerating Medical Solutions
Co-signing Organizations: Prostate Cancer Foundation
Epilepsy Project

Re: Critical Path Initiative (Docket No. 2004N-0181, 69 Federal Register, 21839, April 22, 2004); Establishment of a Docket

Dear Madam/Sir:

FasterCures is a nonpartisan, nonprofit organization whose goal is to save lives by finding ways to shorten the time for discovery, development and deployment of treatments and cures for serious disease. We are independent of any interest or industry groups. Our mission is to evaluate the current system of medical research; identify inefficiencies, misplaced priorities and conflicting incentives that inhibit the pace of discovery and development; and propose and pursue improvements to the existing system. Our focus is on ways to enhance and accelerate the efforts of those involved in creating and overseeing the creation of safe and effective treatments and cures: health, research advocacy and funding organizations; scientists; medical professionals; policy professionals; clinicians; and patients themselves.

Despite the remarkable advances medical research has produced in recent years, cancer, heart disease, diabetes and other illnesses continue to take a staggering toll in treatment costs, lost productivity, suffering and death. By 2001, healthcare spending accounted for more than 14 percent of the gross domestic product (GDP). With 76 million baby-boomers turning 50 at the rate of one every seven seconds, it is imperative that we move research advances forward more quickly. As our population ages, the number of those afflicted by disease and the costs to treat these individuals will rise dramatically in the decades ahead.

The biomedical revolution combined with rapid technological innovation presents the promise of longer life spans and relief from suffering. However, it will take a concerted effort on the part of all of the major participants in the health research endeavor to

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transform those advances into cures. The Food and Drug Administration's (FDA) process through which therapies are approved for marketing in the United States is central to the translation of ideas into medical breakthroughs. And, as the agency itself has recognized, the declining number of drug, biologic and device applications combined with the soaring costs of drug development signals that the system is in trouble.

FasterCures is developing our "Acceleration Agenda" of significant issues impacting the speed with which treatments and cures are discovered and developed. These include:

- ❖ Increasing participation in safe and effective clinical trials;
- ❖ Identifying a technology infrastructure for the management and application of emerging molecular-based information (and other research databases);
- ❖ Encouraging the application of technological innovations to medical research practices; and
- ❖ Promoting the translation of basic research findings into therapeutic breakthroughs.

FasterCures is strongly supportive of and encouraged by the FDA's new Critical Path Initiative. This effort has the potential to contribute to the goal of saving lives by saving time in making new therapies available for use sooner. This potential will be realized only to the extent that the agency pursues this effort in a collaborative way that enlists all relevant stakeholders in defining problems and developing and implementing solutions.

One barrier to faster cures that cuts across all aspects of the medical research and drug development process is the lack of effective cooperation and coordination among the relevant federal agencies, researchers, research institutions, and industry. Successful large-scale scientific collaborations, such as the Human Genome Project, show how important collaboration is. *FasterCures* looks forward to partnering with the FDA in support of such collaborations to achieve the objectives recently laid out in the FDA's report: *Innovation/Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products* (hereafter referred to as the Critical Path Report).

IMPROVING THE CLINICAL TRIALS PROCESS

The FDA—along with the National Institutes of Health (NIH) through its Roadmap initiative—has identified various aspects of the clinical trials process as areas for improvement. In particular, the FDA has identified "an urgent need for improvement in the efficiency and effectiveness of the clinical trials process, including improved trial design, endpoints, and analyses."

We believe there are three aspects of improving the clinical trials process that the FDA should address immediately. First, there is a need to build tools and data repositories that will enable researchers to detect safety problems as early as possible. Second, given current trends toward personalized medicine and smaller markets, the FDA should drive a

process to better define who should be in a given clinical trial (potential responders) so as to identify the drugs most effective for a given population. Third, the FDA should initiate a process to bring disease researchers together to achieve consensus regarding which biomarkers and surrogate endpoints are the most promising to pursue.

We believe that these three areas will not only positively impact the time and cost of clinical trials but also benefit patients. Given the demands that clinical trial participation imposes on patients, not to mention the difficulty and expense of finding enough patients to enroll in clinical trials, particular focus should be given to maximizing the return on every patient's participation in a clinical trial. The proposed initiatives will go a long way towards doing so.

In April of this year *FasterCures*, with the support of U.S. Secretary of Health and Human Services Tommy Thompson, launched *The Patients Helping Doctors (PHD) Program* to highlight and promote the role of patients in the medical research process. Within each patient lies the key to a cure; yet a very low percentage of patients are actively engaged in supporting the research process. This initiative seeks to reverse that trend by educating patients about their critical role in the search for new treatments and cures and by promoting greater fully informed and voluntary patient participation in the medical research endeavor. As *FasterCures* move forward with this initiative, we would welcome the opportunity to work with the FDA to both expand patient participation and ensure that this precious resource is used as effectively and efficiently as possible.

In its Critical Path Report, the FDA addresses the lack of academic programs focused on the medical product development process. *FasterCures* strongly concurs and would point out as well the lack of sufficient numbers of well-trained clinical researchers. Because the FDA holds vital, cross-cutting, and unique knowledge relevant to the clinical research training process, we believe it is essential that the FDA actively participate in efforts to disseminate this valuable knowledge and expertise in building a clinical research workforce appropriate to the growing clinical research needs of the nation.

OPTIMIZING RESEARCH RESOURCES

Limited data access is one of the major obstacles preventing the development of faster cures in the medical research arena. A significant proportion of the data developed, from the investment of billions of dollars in medical research by both the public and private sectors, is neither publicly available nor broadly accessible. Typically, this information is stored in unique, proprietary, stand alone databases that have little or no interoperability with other databases. Broad data access could reduce some of the redundancy inherent in the research process.

The FDA should work actively to find ways to balance the benefits of sharing data with protection for the intellectual property rights of industry and to drive a process to create incentives to increase data sharing within the industry, government, and academic communities. Increasing access to and availability of the FDA's unique datasets has the potential to significantly contribute to therapeutic research. Such an effort could provide

important leadership in promoting and facilitating much broader access to the range of research databases that currently populate the medical research arena.

NEW MODELS OF DISEASE

The FDA's Critical Path Report recognizes the need for better, more accurate, preclinical models of disease given the limitations of our current animal disease models. The report cites several examples where the FDA has led or contributed to the creation of new animal disease models, but does not go on to propose the development of better animal or computational disease models as a focus of the Critical Path Initiative.

Having a greater variety of appropriate disease models available would benefit the development of drugs, biologics, and devices across all categories of disease just as the identification and characterization of the *C. Elegans* nematode and the *D. Melanogaster* fruitfly as new genetic animal models revolutionized the field of genetics and contributed significantly to our understanding of human disease. Through partnering with other governmental and academic scientists, as it has already successfully done, the FDA could promote the development of novel disease models, and in addition, significantly promote the rapid incorporation/utilization of these novel models into the therapeutic development process. This initiative could have profound impacts in "ensuring product safety" and "demonstrating medical utility" by vastly improving the predictive power of preclinical testing, thereby reducing the risk inherent in novel therapeutic development.

MONOTHERAPIES AND COMBINATION THERAPIES

We support the FDA's efforts to develop new tools to expedite and improve the Critical Path to New Medical Products. At the same time we must be sure that we are using current medical products as efficiently and effectively as possible. We encourage the FDA to re-examine the current regulatory approval process for mono- and combination therapies and to make necessary changes to this process to ensure that patients have timely access to the most effective pharmaceuticals. At the same time, we also encourage the FDA to work closely with the Centers for Medicare and Medicaid Services to ensure that approved combinatorial therapies are available as formularies for reimbursement.

DISEASE PREVENTION

As the FDA works on expediting the availability of treatments and cures, we would recommend integrating into that effort a focus on the development of disease prevention tools as well. As highlighted most closely perhaps by the National Cancer Institute's (NCI) current 2015 initiative, disease prevention tools will be some of the most important therapeutic discoveries of the future. Working in partnership with NIH and the Centers for Disease Control and Prevention (CDC), the FDA should promote research on the role of nutrition in disease prevention and create better models of health and early-stage disease. *FasterCures* would be very interested in partnering with the FDA and the NIH to explore mechanisms to increase government, academic and industry interest in and resource allocation towards disease prevention.

VOLUNTARY HEALTH ASSOCIATIONS AND DISEASE FOUNDATIONS

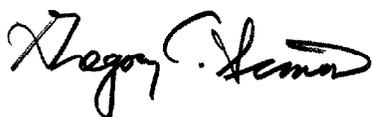
Historically, nonprofit disease foundations have focused on funding basic research and stayed away from issues related to clinical trials and drug development. More and more however, as the gap between basic discovery and later stage development widens, such organizations are focusing on efforts to fill that gap. Although there has been some success in this regard, most groups are operating with little or no information on how to effectively pursue translational research. Developing an outreach effort to provide groups with better access to information and expertise relevant to the critical path process across disease types is one way that the FDA could enhance ongoing foundation efforts in Critical Path initiatives. If even a small portion of the \$3 Billion currently invested by the nonprofit sector in biomedical research efforts were strategically directed into targeted Critical Path initiatives, this could significantly accelerate therapeutic development efforts.

CONCLUSION

In closing, *FasterCures* congratulates the FDA on a thoughtful, informative, and innovative new initiative that has the potential to have long-lasting impacts on the discovery and development process. *FasterCures* is committed to working with the FDA and our other partners in the government, industry, advocacy, and academic sectors to realize this potential. We would welcome the opportunity to work with the FDA in developing and implementing the steps necessary to overcome the obstacles hindering progress on the critical path to successful drug development. Our mission, to accelerate medical solutions, aligns closely with the Critical Path initiatives described in the FDA's recent report and we look forward to partnering with the FDA and others on many of these initiatives in the future.

We thank the FDA for the opportunity to provide comment.

Sincerely,



Gregory C. Simon, JD
President, FasterCures / The Center for Accelerating Medical Solutions

CC: L. Michelson JD, President and CEO, Prostate Cancer Foundation
M. Liang JD, Executive Director, The Epilepsy Project