

July 30, 2004

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

Submitted by email to fdadockets@oc.fda.gov

Re: **Docket: 2004N-0181 - Critical Path Initiative; Establishment of Docket**

Introduction: The Marti Nelson Cancer Foundation is a technical patient advocacy organization, which has worked with FDA in venues including the FDA Patient Consultant - Patient Representative programs. Our comments are outside of the science; rather, they concern process and resources. Thank you for the opportunity to comment on this exciting initiative.

1. *Hurdle Identification. Please describe the product development issue, the nature of the evaluation tool that is out-of-date or absent, how this problem hinders product development, and how a solution would improve the product development process. Please be as specific as possible.*

Hurdle in oncology: Lack of a plan. Oncology product development needs a roadmap with many levels of detail, showing how public and private organizations are going to collaborate to develop better (not just more) tools for the diagnosis, evaluation and treatment of cancer. The NIH Roadmap is a step in this direction; however, it is very broad-brush and not specific to cancer. NCI's Progress Review Groups (PRG) and State of the Science (SoS) meetings come close to this concept, but industry's role is not well-defined, and there is no binding link between PRG/SoS and ongoing public research, let alone incentives for private investment.

For example, the Critical Path document discusses the rich potential of biomarkers in research, diagnosis and treatment. This has also been widely discussed in numerous journals. However, coordination of effort is critical. Lee Hartwell of the Fred Hutchinson Cancer Research Center has given provocative talks (ASCO, AACR) about the potential benefit of biomarkers, along with the need for a coordinated, collaborative effort to bring the potential to reality.

Hurdle in oncology: FDA resources. We have found FDA people to be smart, careful and public-minded. At the same time, there just aren't enough of them to carry the workload. For example, if this project results in specific recommendations and plans, who's going to make it happen? Will it be someone new whose job is dependent on making it work? Or will it be someone who needs to do this on top of an already full plate? Another example ... FDA guidances are rightly identified in the Critical Path document as being valued resources; however, guidance development takes a long time. For example, the FDA - ASCO workshops on surrogate endpoints in oncology have been illuminating; however, they have not yet resulted in a specific guidance to clarify process.

2. *Please rank each hurdle identified in Question 1, above, in priority order according to which hurdles create the most severe product development problems. That is, which problems*

present the greatest opportunity for improving product development processes? Our goal is to identify those aspects of product development that would most benefit from new evaluation tools.

Priority one: Resources– providing dedicated staff whose jobs are dependent on making this work.

Priority two: Roadmap – and if FDA can coordinate and shepherd the biomarker attack, that alone would be a huge public and private benefit.

3. *For each problem identified, please indicate the type of drug, biologic, or device to which the hurdle applies.*

My comments apply only to oncology drugs and biologics, as that is my area of expertise.

4. *For each problem identified, if a solution would facilitate the development of drugs, biologics, and/or devices for a particular disease or categories of disease, please indicate which diseases would be affected?*

Potentially all cancer drugs.

5. *Nature of the Solution. For each problem identified, please describe the evaluation tool that would solve the problem and the work necessary to create and implement the tool/solution. For example, would a solution come from scientific research to develop a new assay or validate a new endpoint? If the solution involves biomedical research, please specify the necessary research project or program. Would a tool be developed through data mining or computer modeling? Would the right tool be a new FDA guidance or industry standard? If work on a solution is underway, what steps remain? Are there other innovative solutions that could be explored?*

Resources: Funding is tough. When we speak with Congress about funding for NIH, we can point to specific programs and initiatives that have succeeded as a result of funding, and plans for the future. This is harder to do with FDA, and we see few advocacy organizations on the Hill pushing on behalf of FDA in the way they push for NIH. Perhaps FDA could work with the advocacy community to explain what's needed to deal with regulation in the 21st century.

Roadmap: See the comments excerpted below from a recent Wall Street Journal article. While this article focuses on use of this roadmap by patients, it will be a valuable resource for both industry and researchers, identifying possible targets and holes that need to be filled.

In addition, the NCI's Clinical Trials Working Group is another "roadmap" area, where FDA's involvement is needed to ensure that public, industry and regulatory concerns are at the table.

Patients Get New Guide To Cancer-Treatment Choices

Government 'Map' Aims To Pull Together Latest Data On Trials and Research

By AMY DOCKSER MARCUS

Staff Reporter of THE WALL STREET JOURNAL

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For cancer patients, one of the most difficult issues following diagnosis is choosing where to get treated and learning what the latest thinking is about their disease.

... Now, in a move with the potential to change the way patients make decisions about their care, the National Cancer Institute and the patient-advocacy group Pancreatic Cancer Action Network are creating a free, Web-based "map" that will track research,

clinical trials and treatment programs related to pancreatic cancer. The goal is to give people a quick, centralized way to weigh their options using the very latest information. The Pancreatic Cancer Research Map, which is being announced tomorrow and launched online in November, aims to list every single clinical trial going on and every relevant researcher, both publicly and privately funded. Available at the PanCAN and NCI Web sites, the map will allow patients to perform personalized searches of trials and research relevant to their cancer sub-type and enable them to find doctors and treatment sites near where they live.

... If the map works as its creators hope, it could become a model for tracking research, trials and key doctors in other cancers, especially rare forms for which information is harder to come by. It could also help patient advocates and cancer researchers determine where gaps in research exist, creating an opportunity to target lobbying efforts for funding.

... For the group's research map, a steering committee of industry, academic, government and patient-advocacy representatives is reviewing scores of grants related to pancreatic cancer. The group expects to have 150-200 government-funded projects listed by November, and is collecting data on privately funded trials to add, as well.

Approximately 115 researchers have signed up for inclusion in the database so far. The map will include contact information about each researcher, as well as abstracts of the researchers' published papers, grant proposals and specialties.

Many pancreatic-cancer patients are diagnosed at very advanced stages of the disease, when it is harder to get into clinical trials. With such detailed information on researchers, the map offers the ability to track down cutting-edge research that may not be listed on Web sites yet. "Patients don't just want more information," says Gloria Petersen, a professor of epidemiology at the Mayo Clinic in Rochester, Minn., and a member of the map's steering committee. "They want to know how the information relates to them and their treatment."

Cherie Nichols, director of NCI's Office of Science Planning and Assessment who serves on the map steering committee, says that "if the map works and is accepted by the community," NCI would consider working with patient groups to develop similar maps for other cancers. "I can see using this model particularly for cancers that are rarer or understudied, where it's harder to find information," Ms. Nichols says.

Write to Amy Dockser Marcus at amy.marcus@wsj.com¹⁸

6. *For each solution identified, please indicate which could be accomplished quickly, in less than 24 months, and which require a long-term approach?*

Resources: While this is a long-term effort, FDA must have current staff dedicated to the success of this initiative and its recommendations.

Roadmap: Specific to cancer, the pancreatic roadmap was done in a relatively short timeframe. Perhaps something similar could be done in other organ sites in a similar timeframe – maybe focusing on the big 4 (lung, colon, breast, prostate).

7. *For each problem identified, what role should FDA play and what role should be played by others? Should FDA play a convening role, bringing the relevant parties together to discuss*

an approach or solution? If so, who else should participate? Should FDA coordinate scientific research, the results of which would be publicly available? We are seeking input on ways to target FDA scientific and collaborative activities to help industry bring more safe and effective medical products to us for review.

Resources: NIH and NCI have both done a good job in this area; perhaps they could offer guidance.

Roadmap: Convening. Have NCI (intramural and extramural), industry and advocates at the table. Bring in leaders who have a track record of getting results, and have staff who will make it happen. Consider involving a past or current member of Congress to help with a political reality-check.

8. *What factors should guide FDA in setting priorities among the hurdles and solutions identified?*

?? Development of **safe, effective and improved** therapies, not just more therapies – therapies that raise the bar rather than lower it. For example, if FDA is reviewing two drugs, one that shows a clear survival advantage and one that shows a response rate, give the survival advantage a higher priority in the queue.

?? Public good, not private good. What's good for industry may not be good for the public.

?? Bringing knowledge together in one spot, to reduce duplication and stretch resources as far as they can go. The Critical Path document rightly identified FDA as a unique repository of knowledge, and I find the FDA presentations at ASCO to be fascinating.

?? Biggest bang for the buck – for example, finding ways to predict tumor response based on tumor characteristics as opposed to organ site.

Thank you for your efforts in this important endeavor. Please feel free to contact us if we can help make this a reality.



Nancy Roach
Chair, Treatment Issues Committee

Address: 2 Eugene Street
Hood River, OR 97031

Phone: 541.386.5476
Email: NancyRoach@CancerActionNow.org

cc: Robert Erwin, President and Founder
Marti Nelson Cancer Foundation