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July 20th, 2010

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

Re: Docket No. FDA-2009-N-0247 (FDA Transparency Initiative: Draft Proposals for Public Comment Regarding Disclosure Policies of the U.S. Food and Drug Administration)

Dear Sir or Madam:

National Patient Advocate Foundation (NPAF) is the voice for millions of patients who have sought care after a diagnosis of a chronic, debilitating or life-threatening illness. The advocacy activities of NPAF are informed and influenced by the experience of patients who receive direct, sustained services from Patient Advocate Foundation (PAF), which provides professional case management assistance to patients with chronic, debilitating or life-threatening conditions. In 2009, PAF resolved 55,384 patient cases and received four million additional inquiries from patients nationally from all 50 states.

On behalf of the patients served by PAF, NPAF is pleased to submit comments to the Food and Drug Administration (FDA) on its FDA Transparency Initiative: Draft Proposals for Public Comment Regarding Disclosure Policies of the FDA. We commend FDA on the approach it has taken with respect to addressing how it can best provide patients and the patient advocacy community with information that affects, or could affect, patient treatment and outcomes. Clearly, the FDA is recognizing the need for a more complete disclosure of information that ultimately benefits patients. This is demonstrated by staffing of the Transparency Initiative's Task Force, which includes Dr. Sharfstein and five of the FDA's key leaders.

NPAF is well aware of the interlocking issues that surround patient care. We view patients as the hub of a wheel with each spoke representing a professional, lay or volunteer service that contributes at varying times to the care scenario. Each of these different services can be affected by disclosure in different ways, and while NPAF supports any disclosure that can benefit patients, we recognize that concern by some over a portion of the 21 draft proposals is equally as important from their perspective.

NPAF appreciates the opportunity to provide the FDA with our unique perspective on the various issues related to FDA's disclosure policies. In the interest of focusing the FDA's attention and resources, we are limiting our comments to those proposals that would have the most direct impact on patients with chronic, debilitating or life-threatening diseases.

At the outset, we would like to note that NPAF supports any FDA efforts to harmonize regulatory requirements and policies with its sister agencies in foreign countries, including most notably the

European Medicines Agency, which currently has in place many of the disclosure policies that the Task Force has proposed. Global harmonization enhances the ability of product sponsors to get innovative treatments to market, and thus to patients, more quickly – a goal that is becoming increasingly important as pharmaceutical and medical device manufacturers develop into large multinational corporations through mergers and acquisitions.

I. Comments Regarding Draft Proposal No. 1

The Transparency Task Force is proposing to expand the amount of information that the FDA makes public about adverse event reports associated with FDA-regulated products. It also is recommending that public information be available in a searchable format that allows users to generate summary reports. As proposed by the Task Force, those summary reports would include the trade name and/or established name of the product, dosage, route of administration, description of adverse event, and health outcome, as well as a clear disclaimer about the limits of the information.

NPAF strongly supports the Task Force's recommendation to increase the accessibility of information on reported adverse events by making the data easily searchable and summarizable. As the Task Force recognized, patients, prescribers, consumers, and public health officials have a particular interest in receiving information about the safety of FDA-regulated products such as prescription drugs and biologics.

The electronic data files released currently via the Adverse Event Reporting System (AERS) are difficult to understand because they are coded; arduous to search because they are formatted as text files; and difficult to use for generating summary reports about a specific product. As a result, those data files are of limited use to patients and consumers who are neither scientists nor informatics specialists. Further, they are of limited use to prescribers and public health officials because obtaining information from the data files is a time and resource-consuming endeavor, and also because the narratives that individual reporters provide to the FDA are not made available. For similar reasons, the existing medical device adverse event report database (MAUDE) is of limited utility to stakeholders and other interested persons.

Accordingly, NPAF encourages the FDA to implement the Task Force's proposal to make adverse event information more accessible to the public through a searchable database that also permits users to generate summary reports of the information. We believe that a system with those features should be put in place for adverse event reports associated with both drugs/biologics and medical devices. Moreover, we consider this proposal significant enough that it should be given high priority by the FDA as it plans how to implement the various disclosure policies that the Commissioner selects in response to the Task Force's final recommendations.

In developing the new system, however, NPAF cautions FDA to ensure that the information is presented in language that a lay person can understand and in language that an individual without a college degree can understand; preferably, the summaries should be written at no more than a sixth-grade reading level. The searchable database as well as the summary reports should not use unfamiliar scientific terms or complex explanations that do not enhance a layperson's understanding of the information being conveyed.

Further, NPAF urges the FDA to be aware of the tone of the summary reports that ultimately will be generated by the system. While we certainly agree that synthesizing information from the various reports received about a particular product is important, the summary reports should not use language that could frighten, inflame, or incite patients and potentially cause them to stop taking a medication without first consulting the prescriber or their treating physician. As NPAF has commented in other forums, the language should not cause disproportionate distress to patients. The summary reports should focus on informing the public in a neutral manner that

simply brings together into a single, cohesive document facts reported to the FDA regarding adverse events associated with a particular product.

II. Comments Regarding Draft Proposal No. 16

The Transparency Task Force is proposing that the FDA disclose relevant summary safety and effectiveness information from an investigational application or pending marketing application “if the Agency concludes that disclosure is in the interest of the public health, which includes when FDA believes it is necessary to correct misleading information” about the investigational or pending product. As the Task Force discussed at length in its report, increased disclosure of information related to the content of pending applications serves many goals, not the least of which is helping patients understand and make decisions about their future treatment options and how best to manage their disease.

NPAF appreciates that the Task Force recognized the importance of striking a balance between public disclosure and the need to ensure that sponsors and manufacturers remain committed to developing innovative products, especially for rare and life-threatening diseases that have few existing treatment options. We certainly agree with the proposition that any new FDA policies should not have an adverse effect on product innovation, but we also consider it imperative that patients enrolled (or considering whether to enroll) in clinical studies have as much information as possible regarding the risks they may be assuming.

In the opinion of NPAF, the Task Force has struck a reasonable balance between the competing interests of persons who favor blanket disclosure of all data from ongoing trials or pending marketing application, and persons who favor the status quo of blanket protection for all such information. A policy of limited disclosure, as the Task Force has proposed, allows important public health benefits to be realized while continuing to respect the rights of sponsors and product developers who have invested significant resources in generating the data and information contained in investigational and pending marketing applications. Those sponsors and product developers have a significant interest in not having their proprietary data released to potential competitors before they have recouped their investments.

In particular, because NPAF fundamentally believes that patients should be empowered to make their own decisions regarding whether to enroll in clinical trials after weighing the potential risks (and potential benefits, if a late-stage trial) of an investigational product, we support the Task Force’s proposal that summary safety and effectiveness information from investigational applications be disclosed in limited circumstances. Similarly, when a marketing application for a new and innovative product is pending review, we consider the release of aggregate information on the safety and effectiveness of the pending product to be important because it would allow patients and their physicians to evaluate the potential for that product to be an improvement over existing treatment options, if indeed any treatment option is even available to patients with rare diseases. We also, therefore, support the Task Force’s proposal that summary safety and effectiveness information from pending marketing applications be disclosed in limited circumstances

As the Task Force has envisioned, the limited circumstances in which such summary safety and effectiveness information would be made public are when FDA concludes that disclosure is in the interest of public health. That would include when the FDA believes such disclosure is necessary to correct misleading information in the marketplace about a product that is the subject of an investigational application or pending marketing application. While those limited disclosure circumstances appear to provide guidelines for the FDA to follow in making a decision regarding the release of summary safety and effectiveness information, NPAF is concerned that they do not offer useful criteria to be applied or clear parameters on FDA’s exercise of its discretion to disclose such information in “the interest of the public health.”

Individuals have diverse ideas regarding FDA's role in protecting the public health, as a result of their training and professional backgrounds, as well as diverse ideas about government transparency more generally. As a consequence, we believe that this disclosure policy could be implemented differently depending on the background of a particular supervisor or even the FDA Commissioner at any given point in time. Such a laudable and important policy, however, should be implemented consistently and fairly regardless of who is in charge of a Division, a Center, or the FDA as a whole.

Accordingly, NPAF recommends that Proposal No. 16 be revised to provide more specific criteria to be applied by the FDA when it is deciding whether to release a summary of safety and effectiveness information from an investigational application or a pending marketing application. The Task Force could develop a non-inclusive list of situations in which disclosure of such information would be in "the interest of the public health." It also could provide examples of the types of "misleading information" that this limited disclosure would be capable of correcting. We believe that incorporating specific examples and possibly so-called case studies into this disclosure policy (should the FDA ultimately adopt and implement it) could potentially reduce the possibility that individual decision-makers would determine that disclosure was not necessary, simply as a result of their professional training or their beliefs regarding government transparency.

NPAF sincerely appreciates the opportunity to share its views on this matter. If we can be of assistance to FDA please do not hesitate to contact us.

Sincerely,

A handwritten signature in black ink, appearing to read "Steve Miller". The signature is written in a cursive, somewhat stylized font.

Steve Miller
Executive Vice President of Regulatory Affairs
National Patient Advocate Foundation
725 15th Street, NW
Washington, DC 20005

CC:
Nancy Davenport-Ennis
President and Chief Executive Officer