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December 16, 2010

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

RE: Docket No. FDA-2010-N-0308 - Parallel Review of Medical Products

Dear Sir or Madam:

National Patient Advocate Foundation (NPAF) would like to thank you for the opportunity to comment on the processes under consideration to establish a Food and Drug Administration (FDA)/Centers for Medicare & Medicaid Services (CMS) parallel review of medical products. NPAF is a non-profit organization dedicated to improving access to healthcare services through both federal and state policy reform. Our mission is to be the voice for 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 case management services from our companion organization, Patient Advocate Foundation (PAF). In fiscal year 2009 (July 1, 2009 – June 30, 2010), PAF resolved 64,188 patient cases and received more than four million additional inquiries from patients nationally.

NPAF recognizes the need for FDA and CMS to integrate their review processes so that novel, effective medical products and devices can efficiently be made available for use by Medicare beneficiaries. Processes that enhance timely patient access to these devices should be considered. It is important that the voluntary parallel review process be used to accelerate the FDA approval/CMS coverage process, since the overarching purpose of parallel review must, in our view, be to move technology forward for the benefit of patients.

NPAF has reviewed the notice and offers the following comments:

Question 2: *For which classes of products would consumers, payers, or sponsors benefit most from parallel review? Why?*

NPAF believes that patients may benefit from parallel review of products and devices that contribute to enhanced development and approval of personalized medicine products. There are multiple barriers that our nation has to overcome as we move into the process of parallel review, including defining a process that allows data collection that addresses the needs of both FDA and CMS to complete the parallel review process, and complications of Medicare reimbursement for companion diagnostic tests. NPAF supports the views expressed by members of Regulatory Education and Action for Patients (REAP) in their comments which cite that personalized medicine products, that include companion diagnostics, are ideal for parallel review. Currently, these

products are burdened by unequal reimbursement for the diagnostic that is required for use in determining if the therapeutic agent is appropriate for the patient. NPAF would refer the agency to the REAP letter submitted to FDA on December 16, 2010, which is a collaborative project of 42 non-profit patient organizations. NPAF is the initiator organization of REAP and collaborated on the comments. The REAP letter is also available on www.npaf.org.

Question 3: FDA and CMS may propose to limit the number of products concurrently under parallel review. How should limits be placed on the number and/or type of products concurrently under parallel review? Should CMS be permitted to review indications for which the sponsor is not seeking FDA clearance or approval under parallel review?

FDA and CMS have the responsibility to review products for safety and efficacy and for coverage and payment, respectively. If the Agencies were to limit the availability of any new parallel review process inappropriately, it could impede more rapid patient access to new therapy options. To avoid this scenario, we suggest allowing preferential access to parallel review for products that display promise in clinical trials and have the potential to bring cutting edge therapies to market. We believe that any established limits on parallel review should not be permitted to delay the “urgent review” of products that promise to bring novel therapeutic benefits to patients. We also would urge the Agencies to review their resource capabilities to assure that any parallel process is sufficiently funded to prevent delay.

Question 4: Are there disadvantages to parallel review?

We believe that a voluntary parallel review process for novel products could prove advantageous to the FDA, CMS, manufacturers, clinicians, and patients if adequate guidance is developed to describe product classes that would benefit from parallel review, to identify agency expectations from interactions with the sponsor, and to reinforce the distinct authorities of the Agencies. Descriptions of product classes that the Agencies believe would benefit from parallel review would allow manufacturers to make key decisions in early development. In order for parallel review to be effective, sponsors must have a clear understanding of each agency’s procedures and both binding and non-binding communications. Finally, it is important to preserve the autonomy and independence of each agency’s decision making process.

Decisions that affect the availability and coverage of FDA-regulated medical products have immediate and long-lasting impact on patients and clinicians who are dependent on product evolution and subsequent approval. FDA and CMS evaluations and policy decisions are complex and probing – as they should be. We believe that a process that takes advantage of the positive aspects of each agency’s current evaluative practices, as well as exploration of new opportunities, can benefit both the Agencies and patients. A renewed and enhanced evaluation process could promote agency efficiencies and enable a novel new product that meets FDA safety and efficacy standards to be made available for coverage under Medicare more quickly. Manufacturers then would be prepared to meet patient demand, knowing early on that CMS has approved coverage and, if that coverage is conditioned on certain criteria, what those criteria are.

We recognize that the statutory limitations on Medicare coverage of FDA-regulated devices mandate that, even under parallel review, FDA marketing approval must precede a NCD issued by CMS. We encourage the Agencies to work with manufacturers in an effort to shorten to the maximum extent possible the lag time between FDA approval and the issuance of a CMS NCD,

where deemed appropriate. Ideally, the two Agencies could announce their decisions essentially simultaneously.

NPAF cautions that any parallel review process that directly or indirectly leads to the potential elimination of Local Coverage Determinations (LCD) would be detrimental to Medicare beneficiaries. Many beneficiaries rely on LCDs for coverage of life-saving therapies and the removal of LCDs would severely affect patient access to these products. Routine use of parallel reviews that result in NCDs could render the LCD process obsolete. This would negatively impact patients because local carriers make coverage determinations consistent with their own respective populations; NPAF continues to support the use of LCDs.

Question 6: Should a voluntary process be put in place to encourage the conduct of clinical trials that are appropriately designed to support both FDA approval/clearance and CMS national coverage decisions? If so, what process should be established?

NPAF supports establishing a voluntary process to encourage the conduct of trials that meet FDA and CMS review standards, as manufacturers are best suited to determine if their products are appropriate for parallel review. Clinical trial policies developed by the Agencies specifically for parallel review would allow manufacturers to better make this determination and would facilitate timely patient access to life-saving devices. Upon establishment of these policies, the Agencies could then publish and solicit comments from interested stakeholders, including manufacturers, in an effort to streamline the clinical trials for all interested parties. Any process that enhances the initiation and completion of clinical trials needed to satisfy both Agencies' data requirements should be considered.

Question 7: What criteria should the FDA and CMS use to decide whether to grant a request for parallel review?

NPAF recommends that the Agencies develop a parallel review process that supports the assessment of promising new therapies and devices. In addition, we recommend that both FDA and CMS agree to a manufacturer's request for parallel review if the manufacturer satisfies the criteria established for parallel review. It may also be noted that manufacturers may be required to provide evidence development criteria in order to achieve coverage.

Question 9: How should parallel review be implemented? Should the agencies use means in addition to a guidance document, such as designating agency liaisons, to educate sponsors about parallel review?

We recommend that FDA and CMS develop guidance documents defining the types of products appropriate for parallel review, identifying the common and unique data requirements for a parallel review, addressing the data confidentiality concerns and detailing the typical procedures, and the appropriate contact points for manufacturers interested in discussing the process. In addition, the Agencies need to take innovative and creative approaches to educate sponsors regarding the parallel review option. It would be helpful if sponsors could utilize a secure web portal to pose questions

and receive answers about parallel review procedures. Interactive formats, such as webinars, are also a very effective communication tool that could be utilized to alert the public about the process option. Agency liaisons could also provide valuable direct agency/sponsor interactions in the context of scientific and public forums. We would urge the Agencies to further define and review agency liaison qualifications and experience requirements if this option is to be implemented. We recommend that the agency liaison pool of constituents include scientists, clinical professionals, patient advocates and retired government agency representatives.

Question 11: Should FDA and CMS have access to the same data and information about the product during parallel review? (Note: Both agencies will protect the confidentiality of proprietary information used in the parallel review process, as they currently do under their respective approval/clearance and coverage processes.)

NPAF recommends that there be mutual agreement between the manufacturer, FDA and CMS to define what data will be mutually shared across both Agencies. Transparency is urged. NPAF applauds the protection of confidential, proprietary information used in the parallel review process. However, without access to the same data, we would suggest that efforts to facilitate parallel review could be delayed, which would negatively affect patient access to these products.

Question 14: Should the agencies convene a joint advisory committee to consider common issues needing public discussion and advice during the parallel review process?

Advisory committees provide independent expert advice to both FDA and CMS as they consider the safety and efficacy of a particular product or device, and subsequently, its coverage. NPAF recommends that a joint advisory committee on parallel review would provide beneficial insight to both FDA and CMS processes. We strongly urge that any such committee include representatives from the patient advocacy, clinical, and research communities as such individuals have proven to be valuable on FDA Advisory Committees as well as CMS' Medicare Evidence Development and Coverage Advisory Committee (MEDCAC). Patients, especially those with chronic, debilitating and life-threatening diseases, are fully engaged in the health care system and provide perspectives that are imperative to the voice of any advisory committee.

Question 15: What other concerns or considerations should the agencies take into account when developing a process for parallel review?

MEDCAC was established to provide independent guidance and expert advice to CMS on specific clinical topics. MEDCAC is used to supplement CMS' internal expertise and to allow an unbiased and current deliberation of "state of the art" technology and science.¹ As the Agencies examine parallel review process structures, we would ask whether consideration has been given to the role of MEDCAC in this process? Specifically, will MEDCAC convene for every product or device that undergoes parallel review? Currently, MEDCAC meets in a public forum approximately six times a year. NPAF proposes that if the Agencies do determine to convene MEDCAC for every device that undergoes parallel review, an evaluation of costs incurred by CMS needs to take place. NPAF would encourage a review by CMS of the role of MEDCAC in the parallel review process and subsequently publish their findings.

Question 16: *Once FDA and CMS have opened a parallel review should a sponsor be able to terminate or withdraw the request for parallel review? If this happens, should that information be made public?*

If the Agencies determine to allow a defined period for withdrawal by the manufacturer of the device from the parallel review process, the Agencies may also determine that there will be a fee associated with this request. NPAF would recommend that if a sponsor elects to have the parallel review process stopped, the determination of what, if any, portion of information would be released to the public would be determined through mutual agreement with the sponsor, the FDA and CMS.

We thank you for the opportunity to comment. We would be pleased to respond to any questions about our recommendations that may arise regarding parallel review in the future.

Respectfully submitted,

A handwritten signature in black ink, appearing to read "Nancy Davenport-Ennis". The signature is written in a cursive style and is positioned above the printed name.

Nancy Davenport-Ennis
Chief Executive Officer and President