Establishment of the FDA Office of Patient Affairs

Policy Proposal:

With the advent of new and innovative patient engagement programs within the Food and Drug Administration (FDA), a growing need for greater coordination of FDA patient outreach is needed. This can be addressed simply: elevate this function to report directly to the Commissioner, equivalent to what has been done with the Offices of Women's Health (OWH) and Minority Health (OMH).

Lifting the current Patient Liaison Program (PLP) out of the Office of Health and Constituent Affairs (OHCA) and Office of External Affairs (OEA) will establish a more visible, central location at the Commissioner level. At this level, the Office of Patient Affairs will coordinate FDA patient engagement by serving as the central resource for patients and patient advocacy groups to proactively engage with the FDA as well as participate within current and future FDA patient involvement opportunities.

The Current Structure:

The PLP was first established in the late 1980's in response to the HIV/AIDS crisis. Staff later transitioned into a permanent office, known as the Office of AIDS and Special Health Issues, and as the office broadened its scope to include all serious and life-threatening diseases, "AIDS" was dropped and it became the Office of Special Health Issues (OSHI).

In addition to serving as FDA's primary liaison to patients and patient advocates, the Office created the Patient Representative Program, giving patients a formal role in regulatory decision-making (e.g., during Advisory Committee meetings). Its location in the Office of the Commissioner allowed it to serve across all of FDA's medical product Centers. In the mid-2000s, OSHI began to liaise with health professional organizations, and subsequently split the Office into the Patient Liaison and Health Professional Liaison Programs.

As FDA became more focused on proactively vocalizing the public health work it was doing, a new office was created: the OEA. This Office drafts the Commissioner's speeches, manages the FDA website and social media, and media relations. In addition, OSHI was reorganized into OEA rather than continuing to report to the Deputy Commissioner and renamed OHCA.

Since that time, with the assistance of the PLP, the number of patient engagement programs within the Centers has expanded considerably.

Because of the Office's increased responsibilities, it is now obvious that with the increased visibility of the Office, and its role as the central coordinator of patient engagement, the mission of the Office must change.

The Need:

The FDA's current structure to ensure the patient's voice is heard within the drug, biologic, and medical device development and review processes must be strengthened in several ways. Most notably, the following opportunities to improve patient involvement should be addressed:

1. **Low visibility of Patient Involvement Opportunities –** Today, many patients are unaware of opportunities to engage the Agency. This could be addressed by FDA more proactively engaging the patient community in FDA patient involvement opportunities. While NORD has worked well with OCHA to include patients at every possible opportunity, many patients and patient organizations are still unaware of those opportunities.

2. **Adverse Conflict-of-Interest Determinations –** Patients, their families, and their caretakers continue to be deemed conflicted under current Federal conflict-of-interest rules due to a misunderstanding of the unique considerations that must be made when reviewing a patient's application.

   This is particularly problematic within the rare disease community, as qualified patients or patient stakeholders may be seen as conflicted due to their attempts to further research and therapeutic development related to their disease. Rare disease communities can be quite small, and often an entire stakeholder community can be judged to be at odds with current laws and regulations, leaving the FDA without a patient representative for an Advisory Committee hearing. Not only does this exclude the patient voice from the process, but it can significantly delay therapeutic development.

3. **Confusion on the FDA's Role in Single-Patient Expanded Access Requests:** The recent passage of state "Right-to-Try" legislation that essentially removes the FDA from the single-patient Investigational New Drug (IND) expanded access request process shows widespread confusion of the FDA's role in granting patients access to investigational therapies. The vast majority of expanded access requests are denied by the sponsor of

the therapy and not by the FDA. In fact, the FDA approves over 99% of single-patient expanded access requests.[1]

This confusion is due at least in part to the lack of a central FDA location for expanded access information and inquiries. Currently, each review division handles single-patient expanded access requests, but the contact information for the appropriate FDA official is difficult to find.

4. **Limited Coordination between Medical Product Centers on Patient Engagement Initiatives:** Each therapeutic review division has a variety of valuable opportunities for patient participation within the drug and device review process. For example, the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) are each taking part in the Patient-Focused Drug Development Initiative.

   The Center for Device and Radiological Health (CDRH) has issued a Draft Guidance on "Patient Preference Information – Submission, Review in PMAs, HDE Applications, and *De Novo* Requests, and Inclusion in Device labeling".[2] Most recently, CDRH formed the Patient Engagement Advisory Council to advise CDRH on the patient's perspective on medical device development and review.[3]

   Each of these initiatives and the many ongoing programs within the current PLP all attempt to include the patient voice in the review process, yet these efforts appear to be disjointed. There is little awareness of patient engagement opportunities across centers, and patient involvement programs are almost entirely self-contained.

The Solution:

Many of these problems can be addressed by simply elevating the current PLP from within the Offices of Health and Constituent Affairs and External Affairs by placing the newly-named Office

[1] Tamy Kim, Peter Laurie, and Richard Pazdur, "US Food and Drug Administration Efforts to Facilitate the Use of Expanded Access Programs" *Journal of Clinical Oncology,* 17 August 2015

[2] U.S. Food and Drug Administration, "Patient Preference Information – Submission, Review in PMAs, HDE Applications, and De Novo Requests, and Inclusion in Device Labeling Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders," http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM446680.pdf (accessed August 28,2015).

[3] U.S. Food and Drug Administration, "Patient Engagement Advisory Committee", http://www.fda.gov/AdvisoryCommittees/CommitteesMeetingMaterials/PatientEngagementAdvisoryCommittee/default.htm

of Patient Affairs (the Office) to the Commissioner level currently occupied by OWH and OMH, for example.

By advancing this Office, patients and patient advocates will become true partners. The voice of patients and patient advocates will strengthen and magnify the patient voice.

The responsibilities of this Office, as outlined by our proposed legislative text, include:

1. **Ensuring Patient Participation Opportunities in the Drug, Biologic, and Medical Device Review Processes:** With the creation of this Office, patients will be better able to take advantage of existing patient involvement opportunities. For example, this Office will more effectively recruit Patient Representatives to serve as Special Government Employees (SGEs).

   This Office will centralize the job of screening patient representative SGE applicants for COI. This Office will then have the authority to grant waivers under 18 U.S.C. § 208. With COI screenings occurring under this Office's purview, patients and caregivers will be reviewed by experts knowledgeable of the patient experience. This could result in a thorough yet expedited review for patient representatives, more a consistent approach to granting waivers.

   An analogous step the FDA has already taken in recognizing and accommodating unique circumstances is the formation of the CDER Office of Rare Diseases. While orphan products are held to the same safety and efficacy standards as other medical products, they require special expertise, knowledge, and often flexibility. Similarly, while patients will have the same protections against conflicted individuals, special expertise and knowledge are needed in assessing their conflict-of-interest determinations.

   This Office will be charged with training and advising SGE candidates. Additionally, the Office will also be responsible for keeping a database of qualified patient representatives to take part in patient involvement opportunities.

   Not only will this elevated Office be valuable in carrying out the FDA's current patient involvement opportunities, but it will also be key in implementing future patient involvement programs, including the language in the 21st Century Cures Act as well as various other FDA patient involvement proposals.

2. **Internal Coordination across the Centers' Patient Engagement Initiatives:** This Office will be tasked with fostering collaboration across Centers on their patient engagement

initiatives. Without usurping the authority of each Center to plan and implement its own patient engagement initiatives, the Office will assist the Centers in coordinating and collaborating, as well as sharing best practices, on patient engagement initiatives.

This can be achieved through the formation of a Patient Engagement Council that will be chaired by the Director of the Office of Patient Affairs. Modeled after the highly successful Rare Diseases Council, this Council will include patient engagement representatives from CDER, CBER, CDRH, CFSAN, OWH, OMH, Offices of Orphan Product Development and Pediatric Therapeutics, and any other office that is deemed appropriate to participate by the Office of Patient Affairs.

This Council will have various duties, including the discussion and sharing of patient engagement and involvement best practices across centers, the development of cross-Center patient involvement opportunities, and the development of patient engagement models to be recommended to the Commissioner and public at large. This Council will also be charged with providing the Commissioner with recommendations for improving patient involvement in the drug, biologic, and medical device development and review processes.

3. **Serve as the FDA's External Representative for Patient Affairs:** This Office will represent the FDA in outside meetings, public appearances, conferences, and other externally facing opportunities for the FDA to proactively publicize patient involvement opportunities. This Office will also be tasked with the development and upkeep of resources for patients to better educate themselves on FDA's regulatory framework, Agency programs and initiatives, and patient involvement opportunities. These resources include, but are not limited to, a patient-centered website and newsletter, similar to the current Patient Network Newsletter.

4. **Educate and Assist Patients and Physicians Seeking Single-Patient Expanded Access Requests:** With better visibility, accessibility, and ability to work across the Centers, this Office will be the sole point-of-contact for incoming single-patient expanded access questions and requests. The Office will include dedicated staff, known as the Expanded Access Coordination Program, to provide education and information to patients and caregivers on the regulatory framework for single-patient expanded access requests, but also to serve as the liaison to the appropriate officials within the Agency for completing the request.

This Office will also keep a database of expanded access requests, the FDA decision on the expanded access request, and the result of the expanded access treatment. This database, which does not exist currently, will allow the FDA to track the results of using an investigational therapy, and will equip Congress and the public at large with data and information on the expanded access environment, something that is currently missing.

This Office will not have any additional regulatory authority on expanded access requests, and the decision to grant a request will remain in the hands of the review divisions. Instead, this Office will simply streamline the process in order to make it more transparent for patients and their caregivers.

5. **Report to the Commissioner on Patient Engagement throughout the Centers:** The Office of Patient Affairs will be responsible for reporting regularly to the Commissioner on all patient engagement and involvement initiatives occurring within the Office and Centers. By elevating this Office to the level directly below the Commissioner, top FDA and Administration officials will be better informed on input solicited from patients and patient advocacy groups, the Centers' individual patient engagement initiatives, the Agency's overarching patient engagement initiatives, and how the Agency and the Administration may stimulate increased patient involvement.

6. **Report to Congress Biennially on Patient Involvement in FDA Practices:** This Office will be responsible for reporting to Congress every two years on the FDA's patient engagement initiatives, and evaluating the progress made within these programs in involving patients. This two-year report should not be overly burdensome on the FDA to produce, and should inform Congress and the general public of opportunities for patients to become involved in FDA practices.

Proposed Legislative Language

A BILL

To improve the safety, efficacy, and security of human medical products and improve the public health through the establishment of the Office of Patient Affairs within the Food and Drug Administration.

Section 1. Short Title

This Act may be cited as the "Food and Drug Administration Patient Affairs Office Act of 2015".

Section 2. Food and Drug Administration Office of Patient Affairs

(a) Establishment– Chapter X of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 391 et seq.) is amended by adding at the end the following:

Section 3. Office of Patient Affairs

(a) Establishment – There is established within the Office of the Commissioner, an office to be known as the Office of Patient Affairs (referred to in this section as the 'Office'). The Office shall be headed by a director who shall be appointed by the Commissioner of Food and Drugs.

(b) Purpose and Duties – The Director of the Office shall –

(1) report to the Commissioner of Food and Drugs on matters related to patient stakeholders, including patients, patient advocates, and their representative organizations;

(2) establish a Patient Liaison Program to serve as a liaison between the Food and Drug Administration (referred to in this section as the 'Administration') and patient stakeholders, which shall include –

(A) provide internal coordination on Administration activities related to patient stakeholder engagement;

(B) assure that patient stakeholder perspectives are taken into consideration during Administration medical product regulatory activities and policy formation;

(C) administer the Patient Representative Program to ensure patient or patient advocate participation in medical product discussions as Special Government Employees in appropriate agency meetings with medical product sponsors and investigators and as voting members of Administration advisory committees for medical products, which includes—

(i) recruit appropriate patient or patient advocate Special Government Employees, through an application process, conflict of interest screening, and determination of whether a waiver may be granted under 18 U.S.C. § 208;

(ii) train Patient Representatives on relevant Administration policies, procedures, and regulations;

(iii) coordinate with Administration centers and offices to include Patient Representatives in appropriate agency meetings with medical product sponsors and investigators and as voting members of Administration advisory committees for medical products;

(iv) maintain a database to track the recruitment and activity of Patient Representatives.

(D) develop and maintain an Administration website for patient stakeholders that provides educational and informational resources related to medical product regulation and other matters related to patients that, no later than [insert month/date/year], shall include a portal for facilitating patient stakeholder input on issues central to the Administration's medical product regulatory activities;

(E) educate and inform patient stakeholders about Administration medical product regulatory activities and policy formation, including through hosting public meetings, administering a newsletter, and attending external meetings and conferences.

(3) establish an Expanded Access Coordination Program to serve as a resource for patient stakeholders, which shall include –

(A) provide education and information to patient stakeholders on the regulatory requirements and Administration's responsibilities for expanded access to investigational medical products, personal importation, and clinical trial participation;

(B) coordinate single patient requests for expanded access with the appropriate Administration centers and offices;

(C) maintain a database to track single patient requests for expanded access.

(4) identify the resources needed to carry out this section and make an estimate each fiscal year.

(c) Council- In carrying out subsection (b), the Director of the Office shall establish a council to be known as the 'Patient Engagement Council' (referred to in this section as the Council').

(1) Chairperson – The Director of the Office shall serve as the chairperson of the Council.

(2) Composition – The Council shall be composed of –

(A) the Director of the Center for Drug Evaluation and Research;
(B) the Director of the Center for Biologics Evaluation and Research;
(C) the Director of the Center for Devices and Radiological Health;
(D) the Director of the Center for Food Safety and Applied Nutrition;
(E) the Director of the Office of Women's Health;
(F) the Director of the Office of Minority Health;
(G) the Director of the Office of Orphan Products Development;
(H) the Director of the Office of Pediatric Therapeutics;
(I) the head of any other Administration office that the chairperson determines is appropriate.

(3) Purposes and Duties – The Council shall –
(A) provide internal coordination and leadership among representative Administration centers and offices, with respect to programs, initiatives, and activities to facilitate patient participation in Administration medical product regulatory activities and policy formation;
(B) provide recommendations to the Commissioner concerning the most pressing issues confronting patient stakeholders in the United States and changes in Administration policy to protect the public health by assuring the safety, efficacy, and security of human medical products, and advancing the public health by helping to speed innovations that make medicines more effective, safer, and more affordable and by helping the public get the accurate, science-based information they need to use medicines to maintain and improve their health;
(C) consider and propose models, policies, and innovative approaches for patient stakeholder participation in Administration medical product regulatory activities and policy formation;

(d) Report – in carrying out subsections (b) and (c), the Director of the Office in collaboration with the Council shall, not later than [insert month/date/year] and biennially thereafter, submit to the Commissioner and publically on the website a report that describes the programs, initiatives, and activities to facilitate patient stakeholder participation in Administration medical product regulatory activities and policy formation, including those in subsection (b) and contains a list of priorities and specific plans for Administration patient stakeholder engagement.

(e) No New Regulatory Authority- Nothing in this section and the amendments made by this section may be construed as establishing regulatory authority or modifying any existing regulatory authority.