

June 12, 2017

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

**Re: Docket No. FDA-2017-N-0455: Enhancing Patient Engagement Efforts Across FDA; Establishment of a Public Docket; Request for Comments.**

Dear Sir or Madam:

On behalf of the millions of men, women, and children in the United States living with common or rare diseases and disabilities, the undersigned organizations thank the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's "Enhancing Patient Engagement Efforts Across FDA; Establishment of a Public Docket; Request for Comments."

We applaud FDA for recognizing the need for a central Office of Patient Affairs to coordinate opportunities for patient involvement in medical product development and regulation at FDA, as well as better assist patients and patient organizations desiring to get more involved. We believe that such an office will be essential to the success of FDA's efforts to enhance patient engagement as outlined in the negotiated User Fee Agreements (UFAs), the Food and Drug Administration Safety and Innovation Act (FDASIA), and the recently passed 21<sup>st</sup> Century Cures Act. However, we also believe that FDA can substantially improve this proposal in several ways, particularly by harnessing patient engagement capabilities already present in the Office of the Commissioner, and by ensuring such an office does not act as a barrier to existing relationships.

It is critically important that this office not hamper or supplant existing relationships between FDA and patients, or patient advocates. The Office of Patient Affairs should not act as a gatekeeper; but should instead serve as a facilitator. If implemented properly, this office would level the playing field; providing an opportunity for the entire patient community to benefit from the level of involvement that some within the community already enjoy.

The proposal under consideration would establish an Office of Patient Affairs to "offer a single, central entry point to the Agency for the patient community." Through triage and navigation services, this office would aim to assist patients in their navigation of engagement opportunities, as well as coordinate patient engagement opportunities across the medical product centers. Additionally, this office would "host and maintain robust data management systems" to build and strengthen relationships with the patient community, as well as improve communication channels for patient stakeholders.

We are appreciative of the attention this proposal affords to improving coordination across the medical products centers, as well as providing a central entry point for the patient community. As it exists currently, many patients are either completely unaware of engagement opportunities, or are only aware of opportunities within a particular review division. This is because opportunities for patient involvement, and the corresponding outreach efforts, are often disjointed, and dispersed across the individual review divisions and medical centers.

With the advent of new and innovative patient engagement programs within FDA, it is more important than ever that there be an office that can provide general coordination both internally and externally for these programs, as well as a straightforward and navigable path for patients to participate. For these reasons, we support creating an office to act as a front door to patients and patient organizations who need assistance navigating the FDA, as well as assist and harmonize the discrete programs already underway in the different medical centers.

It is important to note, however, that the Patient Liaison Program (PLP), housed within the Office of Health and Constituent Affairs (OHCA), already carries out some of the responsibilities related to patient engagement. This office administers, among other things, the highly critical Patient Representative Program. It also coordinates a newly-created patient engagement council of representatives from across the FDA to coordinate and share best practices across the initiatives.

It is our understanding that OHCA would continue to exist if FDA is to pursue the proposal put forward in this notice. Rather than having two centralized offices carrying out partial aspects of patient engagement simultaneously, we strongly believe that FDA and the patient community would be best served by removing OHCA from the Office of External Affairs, positioning it in the Office of Medical Products and Tobacco, and making it the new Office of Patient Affairs.

By proceeding in this manner, as opposed to creating a separate triage office, FDA would ensure that the new Office of Patient Affairs would have the necessary mandate and resources to strategically move patient engagement efforts forward collectively. FDA would also ensure the staff of the new Office of Patient Affairs is already well-educated on, and connected with, the patient community.

With these resources, this office could also address additional concerns related to the patient experience at FDA. Detailed below are three specific areas in which an Office of Patient Affairs could elevate and strategically enhance patient engagement at FDA:

- 1. Confusion on FDA's Role in Single-Patient Expanded Access Requests** – The recent passage of state “Right-to-Try” legislation and consideration of the federal “Right-to-Try” legislation, which together essentially remove FDA from the single-patient Investigational New Drug (IND) expanded access request process, indicate that there is widespread confusion about FDA's role in granting patients access to investigational therapies. This confusion is due at least in part to the lack of a central location within FDA for expanded access information and inquiries. Currently, each review division handles single-patient expanded access requests; however, the contact information for the appropriate FDA official is difficult to identify.

A new Office of Patient Affairs could address this by serving as a central point-of-contact for incoming single-patient expanded access questions and requests. The Office could include a dedicated initiative 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, and also to serve as the liaison to the appropriate officials within the Agency to address the request.

A similar initiative is ongoing within the Reagan Udall Foundation to create an Expanded Access Navigator “aimed at increasing understanding around the issue of individual patient EA to investigational drugs”. While we are encouraged by this effort and wish for it to succeed, we believe there are unique roles that entities within and outside of FDA could serve.

- 2. Adverse Conflict-of-Interest Determinations** – Patients, their families, and their caretakers continue to be deemed conflicted under current Federal conflict-of-interest (COI) rules due to a misunderstanding of the unique considerations that must be made when reviewing a patient’s application. It is also important to note that the less prevalent a disease is, the more challenging it is to identify qualified patients or patient stakeholders. Oftentimes patients are viewed as conflicted due to their attempts to further research therapeutic development related to their disease. There are only so many individuals who can represent particular rare diseases, and it is not unusual for an entire stakeholder community to be determined to be at odds with current laws and regulations, leaving FDA without a patient representative for an Advisory Committee hearing or other FDA-sponsor meetings where patient input would be critical. Not only does this exclude the patient voice from the process, but it can significantly delay therapeutic development. In addition to the regulatory and legal hurdles, review divisions are also tasked with substantial portions of the COI reviews, burdening the staff and disincentivizing them from including patients on the Committees.

A new Office of Patient Affairs could address this by assisting review divisions with conflict-of-interest determinations by conducting the competitor product analysis and alleviating any additional time and resource burdens of conducting these reviews. The Office could also assist in ensuring patient representatives are given COI waivers if there is compelling government interest for their participation.

- 3. Lack of Transparency on Use of Patient Representatives:** Much of FDA’s performance is subject to public reporting; however, program measures for the patient representative program are currently not made publicly available. Some centers and review divisions use patient representatives more frequently than others. In order to understand areas where the program is succeeding and areas where implementation remains low, data on the number of meetings that include patient representatives should be made public through a portal such as “FDA Track,” with categorization by disease area, product type, meeting type and stage of drug or device development.

A new Office of Patient Affairs could address this by reporting regularly to the Commissioner, and to the public, on all patient engagement and involvement initiatives occurring across the entire agency. The new office could also be responsible for reporting to Congress every two years on FDA's patient engagement initiatives, and the progress made within these programs concerning patient involvement. This two-year report should not be overly burdensome on FDA to produce, and should inform Congress and the general public of opportunities for patients to become involved in FDA practices. Additionally, this office could make data available to the public about patient involvement generally at FDA.

In conclusion, we believe this office could greatly improve patient involvement within FDA, and we are hopeful for its creation. However, rather than creating an additional office that could confuse patients and interfere with existing relationships, we believe that repositioning an existing centralized patient office that already administers many of these functions, and has extensive relationships with the patient community, would be the most efficient and effective method for improving patient engagement.

We appreciate the opportunity to comment, and look forward to working with FDA to continue to strengthen patient involvement opportunities.

Thank you in advance for your consideration. For questions on the above comments, please contact Paul Melmeyer with NORD at [pmelmeyer@rarediseases.org](mailto:pmelmeyer@rarediseases.org), or Mark Fleury with ACS CAN at [mark.fleury@cancer.org](mailto:mark.fleury@cancer.org).

Sincerely,

Adrenal Insufficiency United  
Adult Polyglucosan Body Disease Research Foundation  
Allergy & Asthma Network  
Alliance for Patient Access  
Alpha-1 Foundation  
Alport Syndrome Foundation  
American Academy of Dermatology Association  
American Cancer Society Cancer Action Network  
American Lung Association  
American Medical Association  
American Porphyria Foundation  
Amyloidosis Support Groups  
ANDP Kids Research Foundation  
The Association for Frontotemporal Degeneration  
Asthma and Allergy Foundation of America  
Blue Faery: The Adrienne Wilson Liver Cancer Association  
Cancer Support Community  
CARES Foundation, Inc.

CJD Aware!  
The Clarity Foundation  
cureCADASIL Association  
CurePSP  
Cushing Support and Research Foundation  
Cutaneous Lymphoma Foundation  
Desmoid Tumor Research Foundation (DTRF)  
Epilepsy Foundation  
Fabry Support & Information Group  
Family Reach  
Fibrous Dysplasia Foundation  
FORCE: Facing Our Risk of Cancer Empowered  
The Foundation for Prader-Willi Research  
Friedreich's Ataxia Research Alliance (FARA)  
Genetic Alliance Australia  
Global Colon Cancer Association  
Global Healthy Living Foundation  
Global Liver Institute  
The Guthy-Jackson Charitable Foundation  
Hemophilia Federation of America  
Histiocytosis Association  
Hydrocephalus Association  
Hypermobility Syndromes Association  
Immune Deficiency Foundation  
Indian Organization for Rare Diseases  
International Pain Foundation  
International Pemphigus and Pemphigoid Foundation (IPPF)  
International Rett Syndrome Foundation  
Jack McGovern Coats Disease Foundation  
The Leukemia & Lymphoma Society  
Li-Fraumeni Syndrome Association (LFSA)  
Lung Cancer Alliance  
LUNGevity Foundation  
Lymphangiomatosis & Gorham's Disease Alliance (LGDA)  
The Mastocytosis Society, Inc.  
The Michael J. Fox Foundation for Parkinson's Research  
MitoAction  
MLD Foundation  
Moebius Syndrome Foundation  
Morgan Leary Vaughan Fund, Inc.  
The Myasthenia Gravis Foundation of America

The Myositis Association  
National Alliance on Mental Illness  
National Ataxia Foundation  
National MPS Society  
National Organization for Rare Disorders (NORD)  
National PKU News  
National Tay-Sachs & Allied Diseases Association, Inc. (NTSAD)  
NephCure Kidney International  
NGLY1.org  
NICER Foundation  
Oncology Nursing Society  
The Oral Cancer Foundation  
Phelan-McDermid Syndrome Foundation  
The Pitt Hopkins Research Foundation  
Platelet Disorder Support Association  
Prader-Willi Syndrome Association (USA)  
Prostate Cancer Research Institute  
PSC Partners Seeking a Cure  
Rare and Undiagnosed Network (RUN)  
Reflex Sympathetic Dystrophy Syndrome Association (RSDSA)  
RetireSafe  
RZR-1 Foundation  
The Sarcoma Foundation of America  
The Sitosterolemia Foundation  
The Soft Bones Foundation  
Spina Bifida Association  
Spinal CSF Leak Foundation  
SSADH Association  
Susan G. Komen  
Tarlov Cyst Disease Foundation  
Tigerlily Foundation  
Tuberous Sclerosis Alliance  
United Leukodystrophy Foundation  
US Pain Foundation  
The Utah Radon Policy Coalition  
The XLH Network, Inc.  
ZERO - The End of Prostate Cancer