



January 6, 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-2016-N-3462-0001: Establishment of the Patient and Care-Partner Connection; Establishment of a Public Docket;**

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the nearly 7,000 known rare diseases, NORD thanks the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's "Establishment of the Patient and Care-Partner Connection; Establishment of a Public Docket; Request for Comments".

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

We applaud the Center for Devices and Radiological Health's (CDRH) creation of the Patient and Care-partner Connection (P&CC), and thank the Center for its clear dedication to the rare disease patient community.

NORD and its members are grateful for the years of collaborative opportunities and patient-focused initiatives that CDRH has put forward. The CDRH Advisory Committee on Patient Engagement will further facilitate the inclusion of patient viewpoints in CDRH processes; the Patient Preference Initiative has advanced the science of patient input in device development and review, and finally the proposed additions included in the Medical Device User Fee Act (MDUFA) IV Commitment Goals Letter on patient preference information (PPI), and patient-reported outcomes (PROs), will only advance this progress in including patient viewpoints further.

The P&CC, included within CDRH's 2016-2017 Strategic Priorities, is the natural next step in improving patient participation and input into the device development and review process. In order to assist CDRH in the P&CC's creation, we are pleased to submit our proposals in response to the specific questions enumerated in the Request for Comments.

**“What are potential barriers to inclusion for patients and care-partners?” and “What can FDA do to avoid or remedy any barriers to inclusion?”**

NORD foresees several barriers to inclusion for patients and care-partners of which CDRH should be mindful. First, many patients and patient advocates have little to no experience interacting with FDA, or CDRH specifically, and therefore do not have the regulatory expertise necessary to participate in many of FDA collaborative offerings.

While understandable, FDA all too often puts forward esoteric regulatory draft guidances, proposed rules, requests for comments, and other documents that the vast majority of patients and patient advocates cannot adequately discern and comment on. The P&CC can overcome this barrier by ensuring that all information put forward on how patients and patient organizations can participate is accessible and digestible to the full range of regulatory sophistication within the patient community.

Approximately seventy percent of NORD’s 260 member rare disease patient organizations have fewer than five full-time employees. Thus, they likely have limited resources to dedicate to regulatory engagement, developing expertise, or consultants.

Second, even if they have the expertise to participate, they might not have the time as they are responsible for a substantial portion, or perhaps even the entirety, of the organization’s programs. In addition, many, if not most, individual advocates are also the caretakers for a child or adult with a rare disease. If they hope to participate within CDRH opportunities, they must balance this time commitment with all of their other responsibilities.

In crafting participation opportunities, FDA should be mindful of the time constraints faced by most rare disease patient advocates both inside and outside of patient organizations. FDA should craft opportunities for individuals and organizations to participate that cater to the full spectrum of time-constraints.

Third, rare disease patients and their families come from varying socioeconomic backgrounds, but too often the only patients and patient advocates who can participate in FDA offerings are those with the means to take time off from their employment and travel to events and conferences. This prohibits a substantial portion, perhaps even a majority, of rare disease patients and advocates from participating.

We hope FDA considers ways to include patients and advocates from all socioeconomic backgrounds. This should involve developing ways to reach previously excluded individuals through innovative channels. This may also include offering financial assistance to participate.

Finally, many patient advocates, particularly those who are not associated with, or represented by, a patient organization, are simply unaware of opportunities to participate. Even those who have the expertise, time, and resources to participate ultimately may not because word never reached them. FDA can try to address this problem by developing innovative ways to reach out to the patient community and better publicize opportunities for participation.

## **What might patients and care-partners see as appropriate and effective engagement with FDA?**

Rare disease patients and care-partners expect consistent, equitable, and collaborative engagement with FDA. This means that contact information is easy to find and a representative is easy to reach. Patients and care-partners also expect equitable assistance wherein all patients and care-partners, regardless of association or affiliation, are accepted.

Patients and care-partners who participate in input opportunities desire affirmation that their participation, and the time they dedicate to it, is important to FDA and that it is making a difference. We often hear from patients that they participated within FDA programs, and while the act of participation was gratifying, they were not confident that they made any difference. Further FDA attempts to clearly define the outcomes of patient participation will show patients they are making a difference, and will motivate them to continue to participate.

Finally, patients and their care-partners want to develop a relationship with FDA. If they can get to know a particular FDA representative who is responsible for their participation, it will help patients see FDA as a partner.

### **“How appropriate is the program title, “Patient and Care-partner Connection?” and “What, if any, other titles should FDA consider?”**

If CDRH finalizes “Patient and Care-partner Connection” as the title of this initiative, CDRH should be very careful in defining who is invited to participate. The term “care-partner” may specifically discourage many patient advocates from participating as they may interpret this term as pertaining only to those involved in the direct care of a patient. Thus, advocates who are not involved in the day-to-day care of a patient may not see themselves as welcome.

NORD generally uses the term “advocate” as an inclusive term to represent any individual who is not a rare disease patient themselves, but cares about rare disease patients and is in some way involved in trying to improve the rare disease patient experience. This term may be more appropriate for the title of the initiative.

### **“What types of organizations are appropriate for such a partnership?”**

NORD recommends that CDRH broadly includes organizations for which a partnership with FDA could be mutually beneficial. *Bona fide* charitable 501(c)3s that represent patient populations should absolutely be included, and start-up organizations such as support groups, and even social media groups, should be considered for engagement as well. It is important to bear in mind, however, that the process of setting up a 501(c)3 is resource intensive, and groups of patients working together to advance the wellbeing of their patient population will be valuable for FDA to include, regardless of their status.

NORD does caution CDRH against including organizations that are set up by one regulated industry member, and exist to further the interests of that particular company. Their contributions within FDA offerings will likely be biased and unhelpful, and could detract attention from

nonbiased organizations. While there is no simple test that NORD can recommend to CDRH to make this determination, we request that CDRH be mindful of this possibility.

**“What are potential barriers between FDA, partner organizations, patients, and care partners?”**

A current barrier to communication between FDA, partner organizations, patients, and care partners is the dispersal of patient involvement opportunities across FDA with little to no coordination between each initiative. NORD is certainly supportive of the growing number of opportunities for patients to participate within FDA processes, but we are growing concerned about the lack of coordination amongst such opportunities and are worried patients may be confused on how best to participate.

The Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) coordinate the Patient-Focused Drug Development initiative, and CDER’s Professional Affairs and Stakeholder Engagement (PASE) office and Rare Diseases Program (RDP) collaborates with the rare disease patient community. The Office of Health and Constituent Affairs (OHCA) operates the patient representative program and other patient-focused programs. CDRH is already undertaking the creation of the Advisory Committee on Patient Engagement and the Patient Preference Initiative. Both MDUFA and Prescription Drug User Fee Act (PDUFA) proposals also include patient participation opportunities in public meetings and draft guidance development.

The P&CC will ostensibly help coordinate the CDRH patient initiatives, but with all the other patient involvement opportunities across FDA, the environment is still quite confusing for patients attempting to navigate FDA, and discern where best to participate.

To address this problem, NORD proposes that the current Patient Liaison Program (PLP), housed within the OHCA, be elevated to become the Office of Patient Affairs directly beneath the Commissioner (the same level as the Office of Women’s Health and Office of Minority Health).

This location will allow the Office of Patient Affairs to more visibly publicize patient involvement opportunities as well as assist in the coordination of CDER’s PFDD initiative and PASE office, CBER’s patient involvement opportunities, CDRH’s efforts on patient perspective data and the Patient Engagement Advisory Committee, and the various opportunities the PLP already oversees, among others.

This office will be better equipped to handle patient inquiries on expanded access and can better assess patient’s conflict-of-interest determinations. All in all, we believe this office could greatly improve rare and common disease patient’s involvement with FDA, and we are hopeful for its enactment. For more information on this proposal, please [read our full proposal](#).

**How can FDA engage patients, especially those who are hard to reach or from underserved communities who are typically underrepresented in such initiatives?**

This is a routine problem that patient organizations face as well; how do we find patients from underserved communities that we are not reaching? Unfortunately there is no easy answer to this question.

First, we advise CDRH to collaborate with patient organizations, specifically the disease-specific patient organizations, in creating programs to include patients from underserved communities. Second, the P&CC should utilize social media campaigns to reach underserved communities. While these patients and advocates may not be checking the Federal Register or other official avenues FDA uses to communicate with the public, they are likely on Twitter, Facebook, and other social media platforms.

Finally, as recommended above, FDA should consider providing financial assistance to individuals so they can take off work and/or travel to FDA conferences.

### **What lines of questioning would be considered appropriate?**

The rare disease patient community is incredibly altruistic, and given the opportunity, will want to assist other current and future patients. Patients therefore will likely be willing to answer any questions that FDA deems useful if the following conditions are met. First, patients need to be assured that their information and answers are private and secure. Second, patients need to be aware of how their answers will be used, and for what purpose. Finally, patients want to know how their information will impact FDA processes, and how specifically they are making a difference.

If P&CC successfully maintains mutually beneficial, collaborative, and respectful relationships with patients and patient advocates while adhering to the above principles, FDA can expect to develop impactful relationships with the patient community.

### **What characteristics of such a program might patients and care-partners view especially positively and/or negatively?**

Again, patients want to know that their participation, and the time and resources they are investing in participating, is impactful and taken seriously by FDA. NORD frequently talks with patients who are upset that FDA officials did not take them seriously, did not listen to what they had to say, were dismissive of their experience, and offered little to no follow up after meetings.

The P&CC needs to be mindful of these problematic interactions, and work to ensure they do not continue.

### **What methods or qualities of communication might be preferred or convenient for patients and care-partners?**

There are several modes of communication that the P&CC should pursue. First, working through disease-specific patient organizations will help ensure a reasonably representative sample of patients is included. If there are no disease-specific organizations in the space, working through organizations such as NORD to reach the patient population in question would be beneficial.

Creating social media campaigns, and interacting with patients over Twitter and Facebook, as well as other sites where patients congregate (such as Google hangouts and Yahoo groups), will help reach patients that are unaffiliated with established organizations.

Finally, reaching out to physician organizations that represent the physicians who treat rare disease patients is an additional means of communication.

We thank FDA for the opportunity to comment, and we look forward to working with FDA and CDRH in developing this exciting patient involvement opportunity. For questions regarding NORD or the above comments, please contact me at [mrinker@rarediseases.org](mailto:mrinker@rarediseases.org) or (202) 588-5700, ext. 102.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in blue ink, appearing to read "Martha Rinker". The signature is fluid and cursive, with a large initial "M" and "R".

Martha Rinker, J.D.  
Vice President, Public Policy