May 18, 2018

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Re: Docket No. FDA-2017-N-6312-0001: Patient-Focused Drug Development: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data; Public Workshop; Request for Comments

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opportunity to provide comments on the Agency's "Patient-Focused Drug Development: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data; Public Workshop; Request for Comments."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.

NORD has long supported patient involvement in the drug development and regulatory review process. Therapies that are developed and reviewed in consultation with patients are much more likely to reflect the needs and desires of the patient population, and are more likely to offer greater benefits with fewer risks. Only patients who live with the disease can offer these uniquely important perspectives.

Over the course of the last ten years, FDA, often under the instruction of Congress, and in consultation with patients and their representatives, has made great strides in ensuring the patient voice is integrated within the therapeutic development and review process. The Patient Focused Drug Development (PFDD) initiative created by the Food and Drug Administration Safety and Innovation Act (FDASIA) established a series of public meetings in which patients with specific diseases could offer their experiences and perspectives. We believe these meetings were highly successful, and we are already aware of positive outcomes that have resulted.

More recently, the 21st Century Cures Act and the Food and Drug Administration Reauthorization Act (FDARA) further advanced the incorporation of the patient voice in the therapeutic development process. One of the provisions within the 21st Century Cures Act requires FDA to put forward a draft guidance on how the stakeholder community can submit patient experience data to FDA in the form of draft guidance.

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We were entirely supportive of the enactment of this provision within the legislation as we see the submission of patient experience data through draft guidances as one of the more effective methods of bringing empirically-generated patient perspectives to FDA. Several patient organizations within our community, including Parent Project Muscular Dystrophy (PPMD) and the ALS Association, have submitted draft guidances to FDA for consideration.

These draft guidances, and the patient experience data within, can greatly streamline the development and review of orphan therapies for these patient populations. The biopharmaceutical industry can use the patient experience data to ensure their development programs include endpoints and clinical trial structures that reflect patient preferences. The FDA will also have a better understanding of patient viewpoints as they discuss the experimental therapy with the sponsor, and evaluate the safety and effectiveness of the therapy.

To facilitate the most efficient and extensive submission of patient experience data draft guidances to FDA, we suggest FDA include several educational and clarifying provisions within this proposed guidance or accompanying materials.

Appropriate Draft Guidance Generation:

While submitting patient experience data draft guidances to FDA may be an appropriate way to convey the viewpoints of certain rare disease patient communities, it may not be the best way forward for many, if not the majority, of populations within our community.

First, most rare disease patient organizations are quite small. We estimate that over 70 percent of NORD's over 280 patient organization members have fewer than five full-time employees. Given the complexity of collecting patient experience data and submitting it to FDA in the form of a draft guidance, most small rare disease patient organizations will not have the resources to create such products.

Second, even if a rare disease patient organization has the ability and resources to produce patient experience data draft guidances, it may not be the most appropriate time in the drug development process to generate and submit such data. Instead, it may be more appropriate for these organizations to pursue other endeavors to accelerate the development of treatments and cures for their patients.

With this in mind, we hope FDA will include within this proposed guidance advice to the patient community on when submitting patient experience data draft guidances to FDA is most appropriate. We also ask FDA to include information on other, less resource-intensive, options for submitting patient viewpoints to FDA. For example, patient organizations could also consider holding an externally-led Patient Focused Drug Development meeting or listening session, creating a natural history data registry, or nominating a patient for the FDA patient representative program.

It is imperative for FDA to fully communicate through this proposed draft guidance, or elsewhere, that patient experience data draft guidance submission is only one option at the patient organization's disposal to offer their populations perspectives and viewpoints to FDA.

Rubric for Guidance Development:

Patient organizations who choose to pursue developing a draft guidance for FDA will need assistance from FDA on how to construct such guidance, and evaluate the data within. Ideally, FDA could put forward a rubric for guidance development that patient organizations can use to evaluate their draft guidance prior to submission.

This will allow patient organizations to ensure the product they are creating fits the needs of FDA, and will be useful to FDA and industry in therapeutic development and review. FDA could also put forward exemplary existing draft guidance documents as examples so long as FDA ensures that it emphasizes an example draft guidances as only one successful approach, and there may be other methods for patient organizations to pursue.

Audience Clarification:

Patient organizations must be aware that the intended audience for patient experience data draft guidances is not necessarily only FDA, but also the biopharmaceutical industry members who are developing therapies for their conditions. After all, if successful, FDA would take the submitted draft guidance and potentially re-publish it as a "Draft Guidance for Industry".

For this reason, FDA should be entirely clear that the draft guidance should actually be created and formatted with therapeutic developers in mind.

FDA Assistance to Draft Guidance Developers:

Since developing patient experience data draft guidances is not an easy task, it is critical for FDA to offer dynamic assistance to the patient organization community as they collect patient experience data for these purposes. Patient organizations will likely seek assistance from FDA at junctures in their efforts, and FDA should ensure organizations have an accessible team within the Agency to advise the patient organizations as they move forward in their efforts.

Patient organizations will also likely need assistance from FDA on the statistical methodology of collecting patient experience data. Most rare disease patient organizations do not have expert statisticians at their disposal, so additional FDA guidance and assistance may be necessary.

Role of Biopharmaceutical Industry:

We ask that FDA also clarify what an appropriate role for biopharmaceutical industry partners could be within the collection of patient experience data. Many rare disease patient organizations partner with sponsors to advance the development of treatments for their populations. FDA should clarify within this proposed guidance what an appropriate role for a biopharmaceutical partner would be within these endeavors. Patient organizations are always especially careful in ensuring such partnerships are appropriate, and additional assistance from FDA would be helpful.

Related-Disease Organization Partnerships:

Many patient organizations within the rare disease community partner with organizations that represent similar disease populations on large-scale endeavors in order to pool resources and benefit from collective expertise. Rare disease patient organizations may desire to do the same by submitting joint patient experience data to FDA in a draft guidance.

For example, mucopolysaccharidosis (MPS) organizations may desire to partner together to submit data to FDA for all MPS variations. FDA should include instructions within this proposed guidance on when it is appropriate to jointly collect and submit these data, and when these partnerships should not be pursued as they may confound the final product.

We thank FDA for the opportunity to comment and we look forward to working with FDA to ensure rare disease patients and patient advocacy organizations are able to fully participate within this exciting initiative. For questions regarding NORD or the above comments, please contact me at pmelmeyer@rarediseases.org, or 202-545-3828.

Thank you in advance for your consideration.

Sincerely,

Paul Melmeyer
Director of Federal Policy