



March 21, 2019

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

Re: Docket No. FDA-2018-D-4455-0001: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data; Draft Guidance for Industry and Other Stakeholders

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Food and Drug Administration (FDA or the Agency) for the opportunity to provide comments on its draft guidance entitled, “Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data” (“Draft Guidance”).

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 worked closely with Congress and the Agency on the drafting and enactment of section 3002 of the 21st Century Cures Act (Cures Act, Public Law 114–255), which not only enhanced FDA's Patient-Focused Drug Development (PFDD) Initiative but also required FDA to put forward guidance on how stakeholders can submit proposed draft guidance to FDA. We are pleased that FDA is moving forward with the issuance of this guidance and are grateful for the opportunity to provide comments.

Several patient communities within the broader rare disease patient community have already submitted proposed draft guidance to FDA and have found the process and final product to be valuable. For example, the Duchenne Muscular Dystrophy (DMD) community successfully submitted a proposed guidance to FDA that contributed to FDA’s ensuing draft guidance on therapeutic development for DMD. NORD is optimistic this Draft Guidance will lead to an increase in the submission of proposed guidances by other rare disease communities and ultimately accelerate therapeutic development for patients.

NORD participated on a panel at the March 19, 2018 public meeting entitled “Patient-Focused Drug Development: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data.” On this panel, we encouraged FDA to consider several items as it drafted the guidance.

First, NORD encouraged FDA to emphasize that this is just one of many ways in which patients and patient organizations can get involved and that stakeholders should consider the various options before moving forward with any one opportunity. In this vein, NORD suggested that FDA post a decision tree, rubric, or guideline for approaching patient involvement opportunities in order to help guide patient communities towards the opportunity that is best suited for them.

Second, NORD also asked FDA to be mindful of the many rare disease patient organizations with very few resources available to allocate to such an endeavor. NORD encouraged FDA to be as collaborative as possible with these small organizations as they navigate these efforts.

Third, NORD advised FDA to provide greater clarity on the level of granularity or specificity the agency will expect in proposed draft guidances.

Finally, NORD requested that FDA provide a list of best practices or examples of well-prepared and presented proposed draft guidances from other communities to better guide rare patient communities.

NORD is very pleased to see that each of these recommendations is incorporated in this draft guidance. FDA devotes an entire section of the draft guidance to discussing the various patient involvement and data collection opportunities available to patient communities, including when and where each may be most appropriate. FDA also announces the upcoming publication of additional tools (decision tools, flow charts, and templates) on their Patient-Focused Drug Development webpage.

FDA acknowledges the limitations some organizations may have in completing a proposed draft guidance and accommodates these limitations by flexibly approaching the various ways in which patient organizations can present information. FDA also commits to assisting patient organizations as they navigate this process by offering technical assistance just as FDA does for other PFDD efforts.

FDA points to other guidances on collecting patient experience data to answer questions pertaining to the granularity of data collection and cites existing disease-specific draft guidances for those in the patient community to use for content and formatting examples.

We are grateful for FDA’s inclusion of patient registries and natural history data registries in the list of additional ways in which patient communities can collect and submit patient experience data to FDA. As FDA knows, patient-driven natural history data registries hold the promise to greatly increase the understanding of rare diseases by allowing patient communities, therapeutic developers, clinicians, and regulators to better understand the natural progression of rare diseases. NORD’s IAmRare™ platform is currently utilized by 37 rare disease patient

communities, and we hope to greatly expand the program to allow as many rare disease communities as possible to benefit from these advancements.

NORD has two minor suggestions to improve the Draft Guidance. First, while we understand that FDA cannot make any assurances that proposed draft guidances submitted to FDA will have any impact on future FDA activity, this opportunity may be more appealing if FDA were to offer examples of how FDA can use the information submitted by patient communities and how this information has been used historically. Second, FDA does not offer any advice on how patient communities can comment on existing disease-specific guidances that do not have an open comment period. We recommend that FDA suggest ways for our community to be able to do so.

We thank the Agency for the opportunity to comment and look forward to working with FDA on encouraging the rare disease community to take advantage of this new and exciting opportunity. For questions regarding NORD or the above comments, please contact me at rsher@rarediseases.org, or 202-588-5700.

Thank you in advance for your consideration of these comments.

Sincerely,

Rachel Sher
Vice President of Regulatory and Government Affairs