



March 14, 2016

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

Re: Docket No. FDA-2015-N-5106-0001: Clinical Outcome Assessment Compendium

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the nearly 7,000 known rare diseases, NORD would like to thank the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's newly created "Clinical Outcome Assessment (COA) Compendium".

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 are strong supporters of the Patient-Focused Drug Development (PFDD) initiative, and have supported the program since its enactment as part of the Food and Drug Administration Safety and Innovation Act (FDASIA). The PFDD initiative provides rare disease patients with invaluable opportunities to provide their experiences and perspectives to the FDA in order to encourage the development and review of desirable therapeutic options for the rare disease community.

We commend the FDA for the development of the pilot COA Compendium and believe that it may have a substantial positive impact on the research and development of orphan therapies. Rare diseases are particularly difficult to research and develop therapies for due to the general lack of scientific understanding, natural history data, and research attention paid towards these diseases. The creation of the COA Compendium will hopefully provide researchers with guidance on how to proceed in researching therapeutic options for particular rare diseases.

In FDA's request for comments, the Agency specifically requests insights into the utility of the COA Compendium and, "the best approach for developing future iterations of it, including any suggested expansions of its scope". The following are NORD's suggestions addressing these requests.

The Utility of the COA Compendium and Approaches for Future Iterations

NORD believes that the COA Compendium has the promise of facilitating rare disease therapeutic development through the dissemination and publication of difficult to access data. In order to ensure full access and appropriate usage of the COA Compendium, we have several suggestions.

First, to echo several of our patient organization colleagues, we encourage the FDA to provide greater context and clarity to the tool. There is very little explanation contained within the Compendium, and the current iteration may raise more questions than answers on how best to use it. We encourage the FDA to develop a more extensive introduction to the Compendium on how the information should be interpreted, in what context the information is collected, and in what circumstances the information could be used.

We also encourage the FDA to provide materials that are easily understandable and digestible for the rare disease patient organization community. Rare disease patient organizations are often the primary, and sometimes the sole, funder for research into their rare disease. Rare disease patient organizations also often work collaboratively with sponsors in the development and structure of clinical trials. Ensuring that patient organizations of all levels of sophistication can use the compendium will be crucial to its success.

The FDA should also collaboratively work with patient organizations that represent the disease populations contained within the compendium to ensure the information is accurate and up-to-date. Patient organizations are on the front lines of rare disease research, and proactively engaging with rare disease patient organizations will result in a successful product.

We believe it would also be beneficial to describe how this product fits in with the other patient involvement opportunities the FDA offers. For example, the FDA notes that this product should not be seen as a replacement for disease-specific guidance. Further instruction from the FDA on how best to facilitate greater understanding of their disease, such as through guidances, natural history studies, or inclusion within the Compendium, would be helpful to patient organizations that are unsure as to how best to collaborate with the FDA.

Finally, the FDA should ensure that rare diseases are well represented within the Compendium. While the Compendium can only contain the COAs already developed, we encourage the FDA to be particularly vigilant in ensuring all COAs for rare diseases that qualify for inclusion are indeed contained within the Compendium. Currently, only approximately 30 of the over 110 COAs contained within the pilot Compendium are for rare diseases. Given the disproportionate lack of information on rare diseases, the Compendium's significance for rare diseases is especially important.

We thank FDA for the opportunity to comment, and we look forward to working with FDA to ensure the continued growth in therapeutic development for rare diseases, and the continued involvement of patients in the development and review process. For questions regarding NORD or the above comments, please contact me at 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', is written over a light blue rectangular background.

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