July 1, 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-2019-N-1619: List of Patient Preference-Sensitive Priorities; Establishment of a Public Docket; Requests for Comments**

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the Food and Drug Administration (FDA or Agency) for the opportunity to provide comments on the List of Patient Preference-Sensitive Priorities.

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.

The Agency's commitment to meaningful and substantial collaboration with the rare disease community has been unwavering since the enactment of the Orphan Drug Act. It is estimated that there are over 7,000 rare diseases, which are defined in the United States as diseases affecting 200,000 or fewer people. Today, over 90 percent of rare diseases still do not have an FDA-approved treatment indicated to treat the disease. The barriers and significant obstacles that hinder the pursuit of rare disease therapies to meet the substantial unmet medical needs of patients with rare disorders requires the continued partnership of FDA, patients, investigators, and sponsors.

Greater patient involvement in the medical device development and review process is one of NORD's main priorities. We believe that patients and patient organizations need to be fully integrated into the device development and review process in order for more patient-centric devices to reach rare disease patients. We remain supportive of FDA's efforts to incorporate the patient perspective in the development of medical products and regulatory product review. However, NORD believes the proposed Priority List of Patient Preference-Sensitive Areas should include a greater focus on rare diseases. NORD would be happy to work with the Agency to develop a rare disease focused Priority List of Patient Preference-Sensitive Areas.

As we stated in our comments to the Medical Device User Fee Amendments (Docket No. FDA-2016-N-2872) in November 2016, while we understand the need for FDA to focus efforts on the areas of greatest potential benefit, we believe this needs to be done carefully. Too often rare

1779 MASSACHUSETTS AVENUE NW, SUITE 500
WASHINGTON, DC 20036
T 202-588-5700 ▪ F 202-588-5701

55 KENOSIA AVENUE
DANBURY, CT 06810
T 203-744-0100 ▪ F 203-263-9938

1900 CROWN COLONY DRIVE, SUITE 310
QUINCY, MA 02169
T 617-249-7300 ▪ F 617-249-7301

rarediseases.org ▪ orphan@rarediseases.org

diseases have been forgotten due to the small patient populations and limited advocacy resources.

Incorporating a rare disease focus within the Priority List of Patient Preference-Sensitive Areas is consistent with previous FDA statements and could greatly inform FDA's Humanitarian Use Device (HUD) program, which is an alternative pathway for getting market approval for medical devices that helps people with rare diseases or conditions. In FDA's 2016 final guidance, *Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling,* the Agency stated, "patient preference studies can also be informative for devices under HDE [Humanitarian Device Exemption] application review by providing patient perspectives on whether 'the probable benefit to health from the use of the device outweighs the risk of injury or illness from its use, taking into account the probable risks and benefits of currently available devices or alternative forms of treatment.'"[1]

Moreover, during the December 2017 workshop, FDA suggested that patient preference information may be particularly useful, and patient decisions regarding treatment options are preference sensitive, when, for example, patients' views about the most important benefits and acceptable risks of a technology vary considerably within a population or differ from those of health care professionals. This is especially true in the rare disease patient population.

For these reasons, we request that FDA amend the Priority List to incorporate a rare disease focus to ensure that rare disease patients have an opportunity to benefit from this important effort. NORD would be happy to assist the Agency with identifying patient preference-sensitive priorities more specific to rare diseases.

NORD also wishes to respond to the following question posed in this request for comments: "Are there ongoing studies or published studies that adequately address any of these patient preference-sensitive areas in a regulatory context?" NORD's IAMRARE™ Registry Program is playing a critical role in ensuring that natural history data is appropriately and expediently collected and could be used to inform patient preference information for specific rare diseases. In order to facilitate the development of treatments for rare diseases, NORD created the IAMRARE™ Registry Program, with extensive input from FDA, the National Institutes of Health (NIH), patients, organizations, and experts in the field. NORD's platform is an easy to use system that allows patients and organizations to inform and shape medical research and translational science for rare diseases by launching high-quality, customized registries to collect the data needed to define the natural progression of their disease— ultimately advancing product development. Examples of core data elements that are measured within and across each registry at NORD include patient reported, patient experience outcomes related to diagnosis and treatment, quality of life, management of care, clinical testing samples, and clinical reporting. Currently, we are developing natural history studies with twenty rare disease patient groups. We

[1] "Patient Preference Information – Voluntary Submission, Review in Premarket Approval Applications, Humanitarian Device Exemption Applications, and De Novo Requests, and Inclusion in Decision Summaries and Device Labeling." *Food and Drug Administration*, November 2016. https://www.fda.gov/media/92593/download.

believe the data contained within IAMRARE™ Registry could provide valuable information in addressing patient preference-sensitive areas specific to these rare diseases.

We thank the Agency again for the opportunity to comment and look forward to working with FDA to ensure rare disease patients and patient advocacy organizations are able to fully participate within this important effort. For questions regarding NORD or the above comments, please contact me at rsher@rarediseases.org, or 202-545-3970.

Thank you in advance for your consideration of these comments.

Sincerely,

/s/

Rachel Sher
Vice President of Policy and Regulatory Affairs.