



March 27, 2020

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-5646 for “FDA Rare Disease Day 2020: Supporting the Future of Rare Disease Product Development.”

Dear Sir or Madam:

On behalf of the 25 to 30 million Americans with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks the U.S. Food and Drug Administration (FDA) for holding a meeting on February 24, 2020 to commemorate and celebrate Rare Disease Week.

NORD is a unique federation of 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.

It was truly inspiring to know that people around the country came together at events like this one with the shared goal to promote awareness and improve the lives of all people living with rare diseases. NORD was proud to serve as the host and sponsor of Rare Disease Day in the United States, as we have been doing each year since 2009 when our partner organization, EURORDIS, invited us to join the campaign they had started in Europe the previous year. We are looking forward to continuing to participate and to promote awareness for years to come and to expand the reach of NORD's #ShowYourStripes awareness raising campaign. We are so glad the FDA joined in the tradition of recognizing Rare Disease Day by holding this important event.

The purpose of Rare Disease Day is to bring patients and advocates together to articulate with one voice the shared message that millions of people around the world are suffering with unmet medical needs and need help. Our patients need earlier diagnosis; safe, effective treatments; and assured access to medical care and other services.

At the end of February, Rare Disease Day was observed in community settings, government and legislative offices, school classrooms, college campuses, and hospitals. All to make the voices of rare disease patients heard. There were events in state capitol buildings across the nation, where elected officials met with patient advocates to better understand what life is like with a rare disease, and how the health care decisions they make at the state level—on issues such as newborn screening, medical insurance, cost-sharing, and medical foods—have a major impact on

the lives of the approximately 25 to 30 million Americans, or almost 1 in 10 of their constituents, with a rare disease.

It is estimated that there are over 7,000 rare diseases, and over 90 percent of rare diseases still do not have an FDA-approved treatment indicated to treat the disease. As we heard from FDA Commissioner, Dr. Stephen M. Hahn during the FDA Rare Disease Day meeting on February 24, 2020, FDA shares NORD's goal of ensuring that more effective and safe treatments for rare diseases become available.

All the panels at the FDA Rare Disease Day meeting were incredibly informative. And NORD was especially pleased that FDA focused, in part, on natural history registries. As we heard during the meeting, natural history registries offer a unique, exciting opportunity to engage patients in the collection of information about the progression and health impact of a rare disease.

NORD is thrilled to be partnering with the Critical Path Institute (C-Path) on the Rare Disease Cures Accelerator. As both FDA's Dr. Janet Woodcock and Dr. Klaus Romero mentioned, NORD has an important role to play in this partnership. The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) is a robust integrated platform that will include aggregated rare disease data from various sources—including NORD's IAMRARE™ registry platform—and an analytics tool that will allow efficient and effective analysis of data to accelerate rare disease research and inform clinical trial design and regulatory review. Thank you FDA for providing that opportunity.

NORD is a leader in rare disease research and policy. In 2014, NORD launched a patient-driven rare disease natural history study platform called the IAMRARE™ registry program. Dr. Woodcock also mentioned NORD's important work on this initiative during FDA's Rare Disease Day meeting and we are grateful for FDA's recognition. The IAMRARE™ platform was designed with extensive input from FDA, the National Institutes of Health (NIH), patient advocacy organizations, and other public health experts. IAMRARE™ now hosts over forty-five rare disease natural history study partnerships, twenty of which were developed in part through a cooperative agreement with FDA.

The IAMRARE™ registry program works in collaboration with patient advocacy organizations and industry partners to capture natural history data that supports drug development by characterizing and fostering increased understanding of the natural progression of rare diseases and the corresponding burden on patients. This kind of collaboration also exemplifies the topic of the second panel today which underscored the critical importance of collaboration, access to data, and information sharing in the rare disease drug development ecosystem. NORD's IAMRARE™ registry program acts as a resource for patient cohorts for clinical trials, it supports pre- and post-market surveillance, and it fosters the development of clinical outcome assessments. Importantly, the emphasis is on input from the patient and caregiver perspective. NORD only charges a nominal fee to administer a registry, and patients bear no costs for participating.

Even for those rare diseases that do have a treatment, the information obtained through natural history registries offers the potential for better, more effective, more targeted treatments. Armed with information in a registry, researchers and drug developers will be better poised to develop more targeted therapies, or even cures, for patients living with a particular rare disease. With better information about a rare disease, including heterogeneity within the disease, natural history registries also will allow for more effective treatment targets, more specific endpoints, and more efficient clinical trials. We are hopeful that as a result, rare disease patients will get better, more effective treatments, sooner.

The FDA's Rare Disease Day meeting also focused on another critical issue relating to FDA's regulation of "individualized" or "bespoke" therapies. As discussed at the meeting, science has progressed to the point at which therapies can be targeted specifically for a single or a few patients with a specific genetic abnormality. This is a particularly exciting development for rare disease patients who, in many cases, do not have any other treatment options. But this should also be viewed as an exciting development for all patients everywhere: the early clinical work on these small patient populations can lead to a better understanding of both the biology of disease and corresponding effective treatments. Of course, increasing individualization of drug development also raises difficult questions for FDA about how and when the Agency will oversee these efforts. Such questions include how to incentivize these treatments, determine safety and efficacy, scale-up individualized treatments, and ensure affordability. NORD stands ready to work with FDA and the rare disease community to ensure this work on individualized therapies continues with scientific rigor and promotes learning and progress for patients everywhere.

NORD thanks the FDA for supporting Rare Disease Day. We look forward to continuing to partner with FDA in the coming years on innovative initiatives to improve the lives of patients with rare diseases.

Sincerely,

/s/

Michelle Adams

Director of Federal Policy