



NORD Remarks at FDA's Rare Disease Day Meeting February 24, 2020

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 FDA for holding this meeting today to commemorate and celebrate Rare Disease Day.

NORD is a unique federation of health organizations dedicated to helping people with rare diseases through education, advocacy, research, and patient services programs.

NORD is 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.

Rare Disease Day is observed in community settings, government & legislative offices, school classrooms, college campuses, and hospitals. All to make the voices of rare disease patients heard.

It is truly inspiring to know that people around the country are coming together at events like this one with the shared goal to promote awareness and improve the lives of all people living with rare diseases.

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 Dr. Hahn today, FDA shares NORD's goal of ensuring that more effective and safe treatments for rare diseases become available.

All the panels today have been incredibly informative. And NORD is especially pleased that FDA focused, in part, on natural history registries. As we heard this morning, natural history registries offer a unique, exciting opportunity to collect and share information about the progression and health impact of a rare disease.

NORD is thrilled to be partnering with C-Path on the Rare Diseases Cures Accelerator, as both Dr. Woodcock and Dr. Romero mentioned earlier today. Thank you, FDA, for providing that opportunity.

NORD is a leader in this space. In 2014, NORD launched the IAMRARE™ registry program, as Dr. Woodcock also mentioned today.

The platform was designed with extensive input from FDA, NIH, patient advocacy organizations, and other public health experts.

IAMRARE™ now hosts over 40 rare disease natural history study partnerships, 20 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. Importantly, the emphasis is on input from the patient and caregiver perspective.

With better information about a rare disease, natural history registries 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.

Thank you.