July 25, 2014

The Honorable Fred Upton, Chairman
U.S. House of Representatives
Committee on Energy & Commerce
2125 Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton:

These comments from the National Organization for Rare Disorders (NORD) are intended to supplement the record for the hearing entitled “21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication.”

In announcing the hearing, Chairman Pitts said: “Keeping the discovery, development, and delivery cycle of 21st Century Cures constantly improving means ensuring that each phase of that cycle can inform the next. We need to be sure that patients, providers, researchers, and drug and device companies are able to communicate and collaborate in the most productive and transparent manner possible. Patients, for example, know best the real world impact of certain treatments. Taking this information from the delivery cycle and translating it back to those working in the development phase will help ensure that the cycle of cures is constantly improving.”

We at NORD agree on the need for increased communications. There are innumerable communications issues that might be addressed, but in these comments we will focus on two:

1. The need for the patient voice to be heard in the drug development process, and
2. The need for there to be clarity regarding the communication of off-label information, since so many patients with rare diseases use products that are prescribed off-label.

NORD is a unique federation of more than 450 patient advocacy groups, members, and voluntary health organizations dedicated to helping all people with rare diseases. NORD provides resources, research, advocacy, education, community, and infrastructure support to the rare disease community that small individual organizations cannot. NORD’s support allows our member groups to focus on their primary mission, progress towards understanding, treating and curing their diseases.

In the U.S., there are more than 7,000 diseases defined as “rare,” affecting more than 30 million Americans, as well as their families and caregivers. Much progress has been made in public understanding of rare diseases but much more still needs to be accomplished. The vast majority of rare diseases are lacking an approved treatment.
The first issue is how to strengthen the patient’s voice in the drug development and approval process. The Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012 made groundbreaking strides in encouraging that patients play a greater role during the drug approval process. The FDA has implemented many of these changes admirably but there are various other measures contained within FDASIA that are not being implemented to the fullest extent, or not at all. We support efforts to implement existing law concerning patient involvement in the drug development process.

For example, the FDA must include a patient or patient representative on the drug review committee as mandated by section 903 of FDASIA. While the FDA has increased patient involvement in other aspects of the drug approval process, such as in advisory committee meetings, the FDA has yet to include patients on a review panel. NORD requests that FDA be required to fulfill this mandate. Rare diseases are an excellent place to start.

While the FDA has conducted several patient-focused drug development meetings, it has yet to demonstrate how it intends to use the information to inform the drug review process. While NORD appreciates the FDA’s efforts in implementing the patient-focused drug development initiative, we are particularly eager for the findings from these meetings to be incorporated within the drug review process.

NORD requests that the FDA develop a guidance advising patient organizations on how they can administer their own patient-focused drug development meetings and provide data that will be useful to the drug approval process. The FDA is holding 20 patient-focused drug development meetings. The information derived from these meetings can be broadened substantially if FDA provides guidance on how patient organizations can independently conduct their own patient-focused drug development meetings in a manner that would enable the FDA to use the findings of these meetings to enhance the drug review process.

We request that patients be regarded and treated as partners with the FDA in the drug review process. At present, despite progress, patients are regarded as outside participants who are asked to occasionally consult on drug efficacy and effectiveness, usually under the auspices of the drug companies. Specifically, we ask FDA to standardize patient input within the drug review process. Currently, the level of patient involvement varies among review divisions. Patient contribution at regular and predictable times must be built into the process.

The second issue has to do with off-label uses of approved drugs. Many rare disease patients use drugs outside of FDA-approved uses, based on the judgment of their physicians that the drugs will benefit them and will not be harmful.

Recently, patients with rare diseases have been denied reimbursement when they fill a prescription for a drug not specifically approved for their rare disease, but which is approved for other diseases, and from which their doctors believe they will benefit. The insurers are increasingly telling patients with rare diseases that they will not be reimbursed for drugs that their physicians have prescribed for them, if the FDA has not approved the drug for that specific purpose.

The reality is that many drugs are not approved to treat specific rare diseases, even though they are safe and effective for rare disease patients, simply because it does not pay for the
manufacturer to submit an application to the FDA, or to undertake expensive studies. However, many of these drugs are generally accepted as being medically beneficial to rare disease patients, and they should be reimbursed.

At the same time, the government severely restricts what drug companies can say about new research and about off-label uses, thus cutting off information from the most knowledgeable sources. The Congress should seek new policies that permit drug companies to share appropriate information without fear of enforcement action. This will enhance patient and healthcare professional understanding of how approved drugs can be used in the best interests of patients.

We at NORD look forward to continuing to work with the Committee on this important 21st Century initiative.

For questions regarding NORD or the above comments, please contact Diane Dorman, Vice President of Public Policy, at ddorman@rarediseases.org or (202) 588-5700 ext. 102.

Sincerely,

Peter L. Saltonstall
NORD President and CEO