May 30, 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,

On behalf of the 30 million men, women, and children affected by one of the nearly 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) thanks Chairman Upton and the Energy and Commerce Committee for their continuing support of the rare disease community. We also thank you for commencing the 21st Century Cures Initiative, a bi-partisan effort within the House Committee on Energy and Commerce aimed at improving the treatment discovery, development, and delivery process in the United States.

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 welcome the opportunity to comment on the 21st Century Cures Initiative's first white paper titled, "A Call to Action". This white paper raises various questions on how to improve the biomedical innovation cycle and ecosystem, including questions on incentives for drug discovery and development, unnecessary regulatory hurdles within the Federal government, and barriers to accessing treatments once on the market.

In response to these questions, NORD has developed the following legislative concepts. We are excited about the proposals below, and look forward to discussing them with the Energy and Commerce Committee as well as the entire Rare Disease Community. We also recognize that the below concepts represent only a part of the needed reforms to the treatment discovery, development, and delivery cycle for the rare disease patient. We look forward to discussing further ideas as the 21st Century Cures Initiative continues.

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1. Reinstating the Orphan Products Board

To facilitate coordination more effectively among the Federal agencies with jurisdiction over the discovery, development, and delivery of orphan therapies and between these Federal agencies and the rare disease community, NORD recommends that the Committee reinstate the Orphan Products Board within the Department of Health and Human Services. The Orphan Products Board, a now dormant entity in practice but still alive in statute (42 U.S. Code § 236), was established in the Orphan Drug Act in 1983 to "promote the development of drugs and devices for rare diseases or conditions and the coordination among Federal, other public, and private agencies in carrying out their respective functions relating to the development of such articles for such diseases or conditions".

A reinvigorated Orphan Products Board would be beneficial for the entire rare disease community. First, it would facilitate greater communication and collaboration between the Food and Drug Administration (FDA) and the National Institutes of Health (NIH), thus strengthening the bonds between the orphan drug discovery process and the development and approval processes.

Second, a reinvigorated Orphan Products Board would facilitate greater communications between FDA and NIH and the Federal agencies that are instrumental in the delivery of orphan products, such as the Centers for Medicare and Medicaid Services (CMS) and the Department of Defense (DOD). These collaborations will assist in ensuring that critical orphan therapies will actually reach the rare disease patients who need them.

2. Establishing an Office of Clinical Trial Design within the NIH National Center for Advancing Translational Sciences (NCATS)

Clinical trial design is of a paramount importance when developing any therapy, but is especially important for orphan therapies, where innovative trial designs are often needed to accommodate the small disease population. Many companies that are developing orphan therapies are also often small, inexperienced companies that have little practice in designing clinical trials in general, let alone trials for diseases that require an innovative trial design because of factors such as small or geographically dispersed patient populations.

NORD proposes that Congress establish an Office of Clinical Trial Design within the NIH National Center for Advancing Translational Sciences. This office will house some of the foremost experts in clinical trial design, and will consult with sponsors on clinical trial design. In order to motivate sponsors to consult with this newly established office, the FDA must accept the new office's participation in the trial design during the product development process and consider recommendations from that office when determining its approach to reviewing the application for approval of the drug.

This office would also work to ensure strong public/private partnerships in recruitment of patients for clinical trials. Working with this office, patient groups and pharmaceutical sponsors can collaborate on and participate in clinical trial design.

3. Establishing an Office of Clinical Endpoints within the NIH National Center for Advancing Translational Sciences (NCATS)

Similarly as for clinical trial design, establishing an appropriate clinical endpoint can be especially difficult for studies involving rare diseases. All clinical trials must have agreed-upon clinical endpoint(s), intermediate clinical endpoint(s) (ICE), or surrogate endpoint(s) for FDA approval.

NORD proposes the establishment of an Office of Clinical Endpoints within the NIH NCATS to address this issue. Both patient groups and biopharmaceutical companies would be able to consult with this office on clinical endpoints and biomarkers. This office would be helpful in preventing companies and/or patient organizations from spending years and millions of dollars on biomarker research only to receive a rejection from the FDA.

This office would be especially beneficial to the rare disease patient population, as clinical endpoints and biomarkers are particularly difficult to establish within rare, genetic diseases. Similar to the Office of Clinical Trial Design, it is of the utmost importance that the Office of Clinical Endpoints work closely with the FDA, and the FDA factor in this office's involvement and input when assessing potential clinical endpoints and biomarkers.

4. Training of Medical Professionals in Rare Diseases

Currently, the Federal government has various programs to incentivize medical professionals in training to enter certain specialties. NORD proposes that the Federal government establish similar incentives to study and enter fields relating to treating or researching rare diseases. There are various options Congress could take to increase the number of U.S. physicians who are knowledgeable about rare diseases. For example, Congress could implement subsidized training programs within the NIH to encourage research into rare diseases. Congress also could reform the Graduate Medical Education (GME) system to incentivize residency programs on rare diseases.

The U.S. needs more physicians and researchers educated in rare diseases. An increase in medical and scientific professionals with rare disease experience will lead to faster diagnoses, more efficient and effective care, faster discovery of cures, and overall benefits to the health system, as rare disease research will be more easily translated to more common diseases.

5. Establishing a Rare Disease Ombudsman within the Department of Health and Human Services (HHS)

Currently, the rare disease population has representation within both the FDA and the NIH, in the FDA Office of Rare Diseases and Office of Orphan Product Development, and within the NIH at the Office of Rare Disease Research. However, there is no rare disease representation within the parent Department of Health and Human Services, to ensure access to approved products. NORD proposes the establishment of a Rare Disease Ombudsman within HHS to ensure that patients with rare diseases are not subject to barriers in accessing quality coverage that meets their unique healthcare needs. The Rare Disease Ombudsman would:

1. Provide recommendations to the Secretary regarding guidelines on appeals and grievance processes and protections that ensure patients with rare disorders receive access to high quality treatment.
2. Review and advise the Secretary regarding benefit design features critical to patients with rare disorders and unmet medical needs, including, but not limited to, access to prescription drugs, out of pocket costs, and network adequacy.
3. Serve as a single point of contact for patients with rare diseases to address unique issues that impact access to care.

The HHS Rare Disease Ombudsman also would play a role in ensuring that rare disease patients are accessing the necessary care through insurance plans offered under the state marketplaces.

6. Ensuring Access to Orphan Therapies by Addressing Prohibitive Cost-Sharing Structures within both Public and Private Plans

In the 21st Century Cures Initiative's first white paper titled "A Call to Action," the Committee asks, "What uncertainties or barriers currently exist in post-market, real world delivery settings – legal, regulatory, commercial, or otherwise – and how should they be addressed?"

One of the major hurdles in ensuring patient access to orphan therapies is the increased use of high cost-sharing structures within drug plans. These prohibitive cost-sharing structures often involve upwards of 40% co-insurance on drugs placed on the highest tier within the formulary, also known as the specialty tier. These co-insurance requirements require egregious out-of-pocket costs to be paid by the patient on drugs that are extremely expensive in the first place.

There are many times when therapies are not on a plan's formulary. This often results in out-of-pocket limits no longer being applicable, thus subjecting patients to excessive out-of-pocket costs with no cap.

The Energy and Commerce Committee must address this growing trend of pharmaceutical tiering structures with a specialty tier with high co-insurance levels. Even if the Committee is able to improve the drug discovery and development process greatly, as it hopes to do under this initiative, if patients cannot access the drugs due to their prohibitive cost-sharing requirements, the patient experience will not be improved at all.

7. Reforming the Institutional Review Board (IRB) System for Assessing New Therapies

Currently, all clinical trials for new treatments, whether a drug, biologic, or medical device, must receive approval from an IRB. The systems used by IRBs are rarely transparent, and currently there is a gross oversaturation of small IRBs all using different standards, and rarely contributing to the efficacy of the drug. The current system can lengthen the drug development process.

NORD recommends that Congress study the IRB system to see if reforms would allow for treatments to reach patients faster.

8. Creating an "Orphan Protected Class" within the Medicare Part D Program

Recently, CMS proposed the removal of three protected classes from the Medicare Part D drug coverage system. After a unified outcry from the patient population, CMS withdrew the proposal.

NORD acknowledges the need for reform within the Medicare Part D Protected Class system, and would welcome a discussion with CMS with all stakeholders at the table. NORD also proposes that CMS add a Protected Class for orphan therapies. There are rarely alternatives to orphan therapies that patients with rare diseases rely on, yet these drugs are no more protected than any other drug within the Medicare Part D program.

By ensuring coverage of orphan therapies within the Medicare Part D Program, Congress will assure rare disease patients that they will receive the live-saving coverage they need under the Medicare program.

9. Establishing Clearer Federal Policies with Regard to Off-label use of Drugs

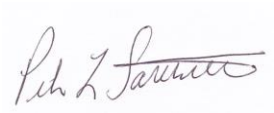
Many rare disease patients use drugs outside of FDA-approved uses, based on the judgment of their physicians that the drugs will benefit them and will not be harmful. Recently, reimbursement for off-label uses has been denied. Congress needs to address this issue aggressively, as many drugs will never be tested for the rare disease patient and, without reimbursement for appropriate off-label use as determined by the physician, these patients will be denied access to approved therapies that may change or save their lives.

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.

Thank you again for the opportunity to engage in this exciting and much-needed initiative. We look forward to working with Chairman Upton and the Energy and Commerce Committee as the 21st Century Cures Initiative continues, and we are grateful for the Chairman's recognition of these extremely important issues within the rare disease community.

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,



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