I want to thank the Food and Drug Administration for providing NORD the opportunity to speak today regarding our position on the reauthorization of the Prescription Drug User Fee Act (PDUFA).

Since 1983, the National Organization for Rare Disorders has served as the voice and advocate for the 30 million men, women and children in the United States affected by one of the 7,000 known rare diseases.

NORD’s mission is to ensure that this nation is one where people with rare diseases can secure access to drugs and biologics that extend and improve their lives, enabling them to be successful members of society. Our objective is to ensure that there continues to be a social, political, and financial culture of innovation that supports both the basic and translational research necessary to create effective therapies for all rare disorders, and supports a regulatory environment that encourages the development of and timely approval of safe and effective treatments for individuals with rare diseases.

NORD views PDUFA V as a unique opportunity to develop a comprehensive series of recommendations to advance orphan product development. The rare disease community believes that engaging Congress and FDA officials in the process will lead to practical, detailed recommendations that can be implemented throughout the development process – from concept to access.

Of significance in the draft agreement is the rare disease initiative that will enhance the development of drugs and biologics for the treatment of rare conditions. NORD supports these efforts and looks forward to the opportunity to work with the Agency to guarantee success of this initiative.

The agreement, as currently written, completes the staffing and implementation plan for the CDER rare disease program within the Office of New Drugs, and establishes a CBER rare disease liaison within the Office of the Center Director.

Among other things, the CBER and CDER offices will develop and disseminate guidance and policy related to orphan product development and work with Center reviewers to improve their understanding of the unique challenges of study design, endpoints and statistical analysis of orphan product development.
Missing in the draft agreement is increased coordination between two other key Centers. Although the regulatory schemes differ between CDER, CBER, CDRH and CFSAN, there are underlying themes of commonality – geographically dispersed small patient populations and, of course, the challenges of trial design. Because NORD’s focus also includes humanitarian use devices and medical foods for inborn errors of metabolism and other rare conditions, increased collaboration and education of reviewers with CDRH and CFSAN is strongly supported by NORD.

**ADVISORY COMMITTEES AND CONFLICT OF INTEREST**

During FDAAA negotiations, NORD argued that because patient populations are very small, few companies are willing to take on the financial risk of developing orphan products, and there are a limited number of researchers conducting this research, identifying experts not financially conflicted to sit on an Advisory Committee would be difficult, if not impossible. Those concerns were realized in 2008 when it took the FDA nearly six months to identify an expert to review a life-saving therapy to treat infantile spasms.

To address those concerns, NORD has joined forces with over 50 organizations who are deeply concerned about the issue of the current conflicts-of-interest statutory provisions and their impact on the appointment of experts, particularly researchers and patients, as Special Government Employees on FDA Advisory Committees and as otherwise needed. As a group, the organizations promote efforts to bring better treatments and cures to those struggling with diseases. Many of these conditions have no adequate treatments and, therefore, it is imperative that we challenge hurdles that impede the quality and efficiency of the treatment development process.

It is our belief that protections must be in place when persons are appointed to positions where their own financial interests might influence their service to the federal government. However, it is also our strong belief that the current conflict-of-interest statutes that apply to the FDA have resulted in a system that is out of balance to the point that conflict avoidance is the primary driver of who serves on Advisory Committees, regardless of the extent of the conflict, the uniqueness of their expertise, or the government’s need for their services.

As you know, FDA SGE’s are subject to an additional layer of statutory conflict-of-interest provisions beyond those that already govern SGE’s for all other departments and agencies in the executive branch. Specifically, under current law, the FDA must analyze potential committee members pursuant to Section 712 of the Food, Drug, & Cosmetic Act (FDCA), in addition to the government-wide provisions found in the Federal Advisory Committee Act and the Ethics in Government Act of 1978. This additional FDA-specific provision appears to drive the FDA to look only for individuals to serve as SGE’s who have virtually no financial ties to any issue that might be addressed by a given Advisory Committee.
While that may sound wise at first glance, in fact those with expertise in a given area often have foreseeable and unavoidable ties to the community as a result of their expertise. Yet, under the current structure, the FDA is not allowing those individuals to serve as SGE’s, despite the fact that by doing so the FDA is being deprived of expertise by those who are best qualified. Accordingly, we support any effort to eliminate the additional conflicts of interest restrictions that apply only to the FDA.\footnote{http://www.accessdata.fda.gov/FDATrack/track?program=advisory-committees&id=AdvComm-waivers&fy=all.}

It is our conviction that the existing provisions in the Federal Advisory Committee Act and the Ethics in Government Act of 1978 are adequate to safeguard against conflicts of interest, while still allowing those with the necessary expertise and perspective to serve on these very important committees. In fact, the specific standard for SGE’s found in 18 U.S.C. 208(b)(3) recognizes that potential SGE’s may have conflicts-of-interest, but allows for their service nevertheless when the need for their services outweighs the potential for a conflict-of-interest created by the financial interest involved. That standard is clear, reasonable, and balanced and appropriately recognizes that some potential SGE’s may come to the FDA with ties to the community that may pose some conflict-of-interest, but that the primary issue must be the government’s need for their services. The main goal of these committees, after all, is to help the FDA to make the best decisions possible. The FDA can only do that if it has the best, most well-informed researchers, clinicians, and patients advising it.

**RISK TOLERANCE IN THE PATIENT COMMUNITY**

Early this year, NORD convened a meeting of like-minded members of the patient community to discuss the willingness or reluctance of patients and their families to tolerate a greater degree of risk in the use of therapies to treat chronic and rare conditions. Our goal was to develop a proposal to be submitted to the FDA as to how the patient community can communicate on a more frequent and periodic basis with medical reviewers and other relevant FDA staff as they are making risk tolerance and other decisions regarding specific product applications or making policy decisions.

The 32 organizations who signed the letter submitted to CDER on September 27 are in full agreement that it is essential that patients have the opportunity to provide such input to product and policy decisions made by the FDA, particularly with regard to risk tolerance associated with the use of specific products. Mechanisms currently exist for patients and other external audiences to provide input to the FDA – e.g., at the public sessions of advisory committees – but the input does not necessarily occur at the time that risk tolerance and other critical issues are being deliberated, and does not necessarily represent a broad spectrum of patient views.
As the FDA commits to a more patient-centric posture, and as patients themselves become more knowledgeable and sophisticated about diseases and their treatment options, we advocate that more systematic processes be established at FDA to enable contributions from the patient community at the time that critical decisions on risk tolerance are being made, and from a representative sample of patient views.

We believe the process should be well-defined and well-understood within the review divisions, and provide a universally applied opportunity for patients to make such input. We are conscious that FDA reviewers and other relevant FDA staff have many demands on their time, but strongly believe that a new process for input will improve product analysis and approval and access to necessary treatments in a timely manner.

We recognize that risk tolerance and other critical decisions are made at many points during the regulatory life cycle of a product - from initial clinical trials through marketing. However, at some points of the review process when risk assessments are made, patient contributions would be of value to the FDA decision-makers.

We recognize continuous interaction with the patient community is not feasible. At the same time, the patient community believes that specific milestone events should be designated at the times at which FDA, as a matter of policy, seeks formal input from the patient community.

We do not seek to create a burdensome or time-consuming process. Rather, we want to be sure that patients across the country, whether they belong to a patient organization or not, have the opportunity to share their views with the FDA.

Our hope and expectation is that the kinds of information that patients and patient organizations can share with the FDA will contribute toward its decision-making in assessing the benefit-risk equation of new products as well as the amount of risk patients at various stages of their condition are willing to take, the quality-of-life challenges they face, the ways they receive information about the proper use of their therapies, how often they see and receive information from their physicians, and other information that FDA medical reviewers and other relevant FDA staff may benefit from knowing directly from patients.

**CLOSING**

In closing, I want to thank the FDA for giving NORD the opportunity to address our concerns related to the reauthorization of the Prescription Drug User Fee Act. We, along with the organizations we have worked with to address conflict-of-interest and risk tolerance issues, welcome the opportunity to work with the Agency.
As a member of the Board of Directors, I would be remiss if I failed to mention the Alliance for a Stronger FDA. The Alliance has over 180 members spanning not-for-profit consumer, patient and research advocacy organizations; associations representing health professions and industry; and individual companies. The Alliance works to ensure annual appropriations that will adequately fund the FDA’s essential missions, and we believe that the American people expect. There is no other agency that performs this critical work. I invite you to learn more about the Alliance and to join forces with it to ensure that the FDA is adequately funded through appropriations.

Thank you.

Respectfully Submitted,

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