Public Meeting - Reauthorization of the Prescription Drug User Fee Program

July 15th, 2015

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Introduction to NORD and Rare Diseases
Facts About Rare Diseases

- There are an estimated 7,000 rare diseases
- 1 in 10 or nearly 30 million Americans have one or more rare diseases
- A rare or “orphan” disease in the US affects less then 200,000 people in a year
- Two-thirds of people with rare diseases are children
- 80% of rare diseases have a genetic component
- Orphan drugs are drugs used to treat rare diseases
- Only about 350 diseases have an FDA approved treatment
Experience of Patients

• It can take years for patients to obtain an accurate diagnosis
• There are limited treatment options for most rare diseases
• Lack of knowledge among many medical professionals
• Treatments are generally more expensive than for regular diseases
• Reimbursement issues related to private insurance, Medicare and Medicaid
Our History

In the decade before 1983, only 10 new treatments were brought to market by industry for diseases that today would be defined as rare.

Leaders of rare-disease patient organizations began to realize that there were certain problems their patients and families shared...problems that were common to all people with rare diseases.

A small story in the LA Times led to an episode on a popular TV show, Quincy ME. Then letters began to arrive from people all over the nation who had rare diseases and thought they were alone in their struggles.

The Orphan Drug Act passed in 1983 and the patient leaders who had worked to bring national recognition to the problem founded NORD as an umbrella organization to represent the rare disease community.

Today, NORD provides information, advocacy, research, and patient services to help all patients and families affected by rare diseases.
Major NORD Programs and Initiatives

- Policy and regulatory advocacy
- State advocacy and alliance partnerships
- Patient representation (FDA, NIH, SSA)
- Education (patients, professionals, public)
- Mentoring (patient organizations)
- Patient assistance programs
- Patient Networking (disease specific meetings, online communities, creation of new patient organizations)
- Increase disease understanding (Research grants, patient registries)
- International Partnerships
- US Sponsor of International Rare Disease Day
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  - Greater coordination across centers on patient involvement
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• Strengthen and incorporate the patient voice throughout the drug development process
  – Patient-Focused Drug Development
  – Participation on Advisory Committees
  – Greater coordination across centers on patient involvement
  – Inclusion of the patient voice throughout the development process, not just the end of the review
Goals for PDUFA VI

• Ensure orphan incentives remain strong
  – Strengthen the Rare Pediatric Disease Priority Review Voucher program
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• Promote consistency across review divisions on the use of expedited review pathways as well as biomarkers and surrogate endpoints for rare diseases
Thank You!

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